

# minneapolis

The American Society of Gene Therapy's 7th Annual Meeting

MN MINNEAPOLIS  
JUNE 2-6, 2004

# final program

# The American Society of Gene Therapy's 7th Annual Meeting final program

# 2004

## Table of Contents

Welcome .....	2
Committee Meetings and Committee Rosters .....	3
Annual Meeting Supporters .....	9
Annual Meeting Exhibitors .....	10
Abstract Reviewers .....	11
Planning Committee and Group Coordinating Reviewers .....	12
Research Awards and Travel Grant Recipients .....	13
Faculty Listing .....	14
General Meeting Information .....	20
Minneapolis Convention Center Floor Plans .....	24
Housing Information and Map of Minneapolis .....	27
Program-at-a-Glance .....	28
Program Schedule .....	29
Abstract Directory .....	47
Abstract Author Index .....	119
Exhibit Hall Floor Plan .....	145
Exhibitor Information .....	146

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004



Dear Colleagues,

On behalf of the American Society of Gene Therapy, I am pleased to welcome you to the ASGT 7<sup>th</sup> Annual Meeting. This year's exceptional program includes the George Stamatoyannopoulos lecture by Harriet L. Robinson, PhD, a Presidential Symposium presentation by Gordon Keller, *Deriving Tissues from Embryonic Stem Cells*, and Catherine Verfaillie, *Unexpected Potency of Adult Stem Cells: Fact or Fiction?*; 21 education sessions; 13 state-of-the-art scientific symposia; 20 workshops; oral and poster sessions selected from submitted abstracts; Meet-the-Investigator sessions; and two corporate symposia.

I would like to thank the Society's committees for their hard work and diligence for putting together an outstanding program. The numerous topics that will be covered include genetic therapy approaches to cancer, cardiovascular disease, genetic diseases, hematopoietic cells, infectious disease, stem cells, musculo-skeletal disorders, neural disorders, non-viral gene transfer, viral gene transfer, respiratory tract disorders, and bioethics. There will also be a special workshop on international harmonization of regulatory standards.

I would like to thank our exhibitors and commercial supporters for their participation and generosity, as well as our world-renowned faculty who agreed to share their expertise and time.

This is a very exciting time for the American Society of Gene Therapy. The Society continues to grow and this forum is the perfect opportunity for us to share developments with our colleagues. I welcome you to Minneapolis.

Sincerely,

Donald B. Kohn, MD  
President, ASGT

### ASGT OFFICERS & DIRECTORS

#### OFFICERS

##### **President**

Donald B. Kohn, MD  
Childrens Hospital Los Angeles

##### **Incoming President**

Katherine A. High, MD  
The Children's Hospital of Philadelphia

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Targeted Genetics Corporation

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Editor-in-Chief, *Molecular Therapy*  
(ex-officio)  
  
Savio L.C. Woo, PhD (04)  
Chair, Advisory Board  
(ex-officio)

### ASGT Executive Office

555 East Wells Street  
11<sup>th</sup> floor  
Milwaukee, WI 53202-3823  
Phone: 414-278-1341  
Fax: 414-276-3349  
Email: [info@asgt.org](mailto:info@asgt.org)  
[www.asgt.org](http://www.asgt.org)

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Director of Meetings &  
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# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Committee Meetings • ASGT 7th Annual Meeting

Committee terms technically begin and end in June at the Annual Meeting. Incoming chair and committee member terms officially go into effect on June 4, 2004, at the Business Meeting. A complete committee roster is included for your reference. Current members of committees are welcome to attend their committee meetings.

THURSDAY, JUNE 3	
12:30 pm – 1:30 pm	Publications Committee
12:30 pm – 1:30 pm	Education Committee
4:30 pm – 5:45 pm	Clinical & Regulatory Affairs Committee
FRIDAY, JUNE 4	
12:15 pm – 1:30 pm	Industrial Liaison Committee Meeting
12:15 pm – 1:30 pm	Ethics Committee Meeting
5:15 pm – 6:30 pm	2005 Planning Committee Meeting
5:15 pm – 6:30 pm	International Committee
SATURDAY, JUNE 5	
7:00 am – 8:00 am	Scientific Committee Meetings
12:15 pm – 1:15 pm	Scientific Committee Meetings
12:15 pm – 1:15 pm	Membership Committee Meeting
12:15 pm – 1:15 pm	Advisory Board Meeting



### Society Annual Business Meeting

*The ASGT Business Meeting will take place in Room 213 of the Minneapolis Convention Center on Friday, June 4<sup>th</sup>, from 4:30 pm to 5:15 pm. All ASGT*

*members in good standing are welcome to attend. Election results will be announced, new officers will be installed, and the Society's finances and membership statistics will be reviewed. We welcome your input and participation.*

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Committee Roster

*Terms begin and end at the June 7<sup>th</sup> ASGT Annual Business Meeting (through June 2004)*

#### STANDING COMMITTEES

##### Advisory Board

*Chair*

Savio L.C. Woo, PhD (2002 - 2004)

*Members*

David Bodine, PhD (2003 - 2008)

Malcolm Brenner, MD, PhD (2002 - 2007)

Theodore Friedmann, MD (2000 - 2005)

Fred Gage, PhD (1999 - 2004)

Joseph C. Glorioso, PhD (2003 - 2008)

Richard C. Mulligan, PhD (2001 - 2006)

Alan E. Smith, PhD (2002 - 2007)

Inder Verma, PhD (2001 - 2006)

James M. Wilson, MD, PhD (1999 - 2004)

##### International

*Chair*

Odile Cohen-Haguenauer, MD, PhD (2002 - 2004)

*Members*

Fatima Bosch, PhD (2002 - 2005)

Yoshikatsu Eto, MD, PhD (2001 - 2004)

David Klatzmann, MD, PhD (2003 - 2006)

Xi-Zhang Lin, MD (2001 - 2004)

Margaret A. Liu, MD (2001 - 2004)

Philippe Moullier, PhD (2004 - 2007)

Keiya Ozawa, MD, PhD (2003 - 2006)

John Rasko, MD, PhD (2004 - 2007)

Felicia M. Rosenthal, MD, PhD (2002 - 2005)

Ning-Sun Yang, PhD (2001 - 2004)

##### Clinical and Regulatory Affairs

*Chair*

Terence R. Flotte, MD (2002 - 2004)

Daniel R. Salomon, MD (2004 - 2005)

*Members*

Estuardo Aguilar-Cordova, PhD (1998 - 2004)

W. French Anderson, MD (2001 - 2004)

Dale Ando, MD (2002 - 2005)

Arthur L. Beaudet, MD (2004 - 2007)

Timothy Billiar, MD (2002 - 2005)

Malcolm Brenner, MD, PhD (2000 - 2004)

Kenneth Cornetta, MD (1998 - 2006)

Terence R. Flotte, MD (2002 - 2005)

Carl H. June, MD (2004 - 2007)

Robert W. Martin (2003 - 2006)

R. Scott McIvor, PhD (2002 - 2005)

Andra Miller, PhD (2002 - 2005)

Glenn Pierce, MD, PhD (2004 - 2007)

Greg Podskaloff, MD (2003 - 2006)

Daniel R. Salomon, MD (2003 - 2006)

Stephanie Simek, PhD (2003 - 2006)

##### Education

*Chair*

Matthew Weitzman, PhD (2003 - 2005)

*Members*

Estuardo Aguilar-Cordova, PhD (2004 - 2007)

Jeffrey Bonadio, MD (2001 - 2004)

Kenneth Cornetta, MD (2003 - 2006)

Olivier Danos, PhD (2004 - 2007)

Beverly L. Davidson, PhD (2003 - 2006)

Patrick Kelly, MD (2002 - 2005)

Philip L. Leopold, PhD (2004 - 2007)

Fulvio Mavilio, PhD (2004 - 2007)

Katherine Ponder, MD (2002 - 2005)

##### Ethics

*Chair*

Theodore Friedmann, MD (2000 - 2004)

R. Scott McIvor, PhD (2004 - 2005)

*Members*

Jean Bennett, MD, PhD (2002 - 2005)

Lawrence C. Chan, MD (2004 - 2007)

Ronald G. Crystal, MD (2001 - 2004)

David A. Dichek, MD (2001 - 2004)

Cynthia Dunbar, MD (2004 - 2007)

Theodore Friedmann, MD (2000 - 2005)

Dieter Gruenert, PhD (2003 - 2006)

Margaret A. Liu, MD (2002 - 2005)

Punam Malik, MD (2004 - 2007)

Gerard McGarrity, PhD (2001 - 2004)

R. Scott McIvor, PhD (2001 - 2004)

Catherine McKeon, PhD (2001 - 2004)

Seppo Yla-Herttula, MD, PhD (2002 - 2005)

##### Financial Affairs

*Chair*

Alan E. Smith, PhD (2000 - 2004)

*Members*

Xandra Breakefield, PhD (2002 - 2005)

Douglas J. Jolly, PhD (2001 - 2004)

Inder Verma, PhD (2001 - 2004)

**Roster as of April 23, 2004**

# The American Society of Gene Therapy's 7th Annual Meeting

## final program



### Committee Roster

*Terms begin and end at the June 7<sup>th</sup> ASGT Annual Business Meeting (through June 2004)*

#### Industrial Liaison

##### *Chair*

Parris Burd, PhD (2004 - 2005)

Stephen M.W. Chang, PhD (2002 - 2004)

##### *Members*

Parris Burd, PhD (2001 - 2004)

Barrie Carter, PhD (2001 - 2004)

Joy Cavagnaro, PhD, DABT, RAC (2002 - 2005)

Ken Chahine, PhD (2004 - 2007)

Boro Dropulic, PhD (2003 - 2006)

Mitchell Finer, PhD (2001 - 2007)

Anthony Green, PhD (2003 - 2006)

Richard Gregory, PhD (2001 - 2004)

Douglas J. Jolly, PhD (2003 - 2006)

C. Richter King, PhD (2002 - 2005)

Edward Lanphier (2004 - 2007)

Russette M. Lyons, PhD (2002 - 2005)

Robert C. Moen, MD, PhD (2001 - 2004)

John Monahan, PhD (2003 - 2006)

Ralph W. Paul, PhD (2004 - 2007)

Leonard E. Post, PhD (2000 - 2006)

Alain Rolland, PharmD, PhD (1999 - 2006)

Dominick A. Vacante, PhD (1999 - 2004)

Samuel C. Wadsworth, PhD (2004 - 2007)

Peter K. Working, PhD, DABT (2002 - 2005)

#### Membership

##### *Chair*

Paul D. Robbins, PhD (2003 - 2005)

##### *Members*

C. Anthony Blau, MD (2004 - 2007)

Seng H. Cheng, PhD (2004 - 2007)

J. Victor Garcia-Martinez, PhD (2003 - 2006)

Mark A. Kay, MD, PhD (2002 - 2005)

Qiang Liu, PhD (2003 - 2006)

Richard Morgan, PhD (2001 - 2004)

Isabelle Riviere, PhD (2004 - 2007)

Louis C. Smith, PhD (2001 - 2004)

Clifford J. Steer, MD (2001 - 2004)

Sean M. Sullivan, PhD (2001 - 2004)

#### Nominating

##### *Chair*

Arthur Nienhuis, MD (2003 - 2004)

##### *Members*

Malcolm Brenner, MD, PhD (2003 - 2004)

Seng H. Cheng, PhD (2003 - 2004)

Odile Cohen-Haguenauer, MD, PhD (2003 - 2004)

Beverly L. Davidson, PhD (2003 - 2004)

#### Program

##### *Co-Chairs*

Malcolm Brenner, MD, PhD (2002 - 2004)

Robert L. Martuza, MD (2002 - 2004)

##### *Chair*

Arthur Nienhuis, MD (2004 - 2005)

##### *Vice Chair*

Michel Sadelain, MD, PhD (2004 - 2005)

##### *Members*

Donna Armentano, PhD (2001 - 2004)

David Bodine, PhD (2001 - 2004)

Michele P. Calos, PhD (2003 - 2006)

Barrie Carter, PhD (2001 - 2004)

David T. Curiel, MD, PhD (2001 - 2004)

Mary Dinauer, MD, PhD (2001 - 2004)

Philip R. Johnson, MD (2004 - 2007)

Luigi Naldini, MD, PhD (2004 - 2007)

David W. Russell, MD, PhD (2001 - 2004)

Stephen J. Russell, MD, PhD (2002 - 2005)

Matthew Weitzman, PhD (2004 - 2007)

Jon Wolff, MD (2001 - 2004)

#### Publications

##### *Chair*

Jeffrey Chamberlain, PhD (2001 - 2004)

George Stamatoyannopoulos, MD (2004 - 2005)

##### *Members*

W. French Anderson, MD (2003 - 2007)

David Bodine, PhD (2004 - 2007)

Barrie Carter, PhD (2003 - 2006)

Jeffrey Chamberlain, PhD (2001 - 2005)

Theodore Friedmann, MD (2001 - 2004)

Katherine A. High, MD (2003 - 2006)

Brendan Lee, MD, PhD (2004 - 2007)

Pedro Lowenstein, MD, PhD (2002 - 2005)

Michel Sadelain, MD, PhD (2001 - 2004)

# The American Society of Gene Therapy's 7th Annual Meeting final program



## Committee Roster

*Terms begin and end at the June 7<sup>th</sup> ASGT Annual Business Meeting (through June 2004)*

### SCIENTIFIC COMMITTEES

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##### *Chair*

David T. Curiel, MD, PhD (2003 - 2004)

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##### *Members*

Steven M. Albelda, MD (2004 - 2007)

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Michael Jensen, MD (2003 - 2006)

Robert L. Martuza, MD (2001 - 2004)

John Morris, MD (2003 - 2006)

John J. Nemunaitis, MD (2003 - 2006)

Richard G. Vile, PhD (2004 - 2007)

Richard J. Whitley, MD (2002 - 2005)

James Zwiebel, MD (2003 - 2006)

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##### *Chair*

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Anthony Rosenzweig, MD (2004 - 2005)

##### *Members*

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Timothy Billiar, MD (2002 - 2005)

Barry J. Byrne, MD, PhD (2001 - 2004)

Lawrence C. Chan, MD (2000 - 2005)

J. Kevin Donahue, MD (2004-2007)

Victor Dzau, MD (2003 - 2006)

Arthur Feldman, MD, PhD (2002 - 2005)

Frank J. Giordano, MD (2001 - 2004)

Roger J. Hajjar, MD (2004 - 2007)

Ralph Kelly, MD (2003 - 2006)

Douglas Losordo, MD (2003 - 2006)

Todd K. Rosengart, MD (2001 - 2004)

Anthony Rosenzweig, MD (2003 - 2006)

Sonia Skarlatos, PhD (2004 - 2007)

#### Embryonic Stem Cells & Tissue Engineering

##### *Chair*

Gordon Keller, PhD (2003 - 2005)

##### *Members*

Mark E. Davis, PhD (2003 - 2006)

Stanton L. Gerson, MD (2003 - 2006)

Darwin J. Prockop, MD, PhD (2004 - 2007)

Mervin Yoder, MD (2003 - 2006)

#### Gene Therapy of Genetic Diseases

##### *Co-Chairs*

Jean Bennett, MD, PhD (2003 - 2005)

Katherine Ponder, MD (2004 - 2005)

##### *Members*

W. French Anderson, MD (2001 - 2004)

Seng H. Cheng, PhD (2004 - 2007)

Markus Grompe, MD (2003 - 2006)

Eric Hoffman, PhD (2002 - 2005)

Mark A. Kay, MD, PhD (1999 - 2004)

Philippe Leboulch, PhD (2003 - 2006)

Brendan Lee, MD, PhD (2004 - 2007)

Katherine Ponder, MD (2003 - 2006)

Judith St. George, PhD (2002 - 2005)

James M. Wilson, MD, PhD (2001 - 2004)

Joseph Zabner, MD (2004 - 2007)

#### Gene-Based Vaccines

##### *Chair*

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Margaret A. Liu, MD (2003 - 2004)

##### *Members*

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Stephen L. Eck, MD, PhD (2003 - 2006)

Hildegund Ertl, MD (2003 - 2004)

Philip R. Johnson, MD (2003 - 2006)

Carl H. June, MD (2004 - 2007)

Art Krieg, MD (2003 - 2006)

Patrick Squiban, MD (2003 - 2006)

Britta Elisabeth Wahren, MD, PhD (2003 - 2006)

David B. Weiner, PhD (2003 - 2006)

Ning-Sun Yang, PhD (2003 - 2006)

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##### *Chair*

John E. Dick, PhD (2002 - 2004)

Jan Nolta, PhD (2004 - 2005)

##### *Members*

David Bodine, PhD (2000 - 2006)

Fabio Candotti, MD (2004 - 2007)

Gay Crooks, MD (2002 - 2005)

Mary Dinauer, MD, PhD (2003 - 2006)

Robert G. Hawley, PhD (2004 - 2007)

Helen E. Heslop, MD (2002 - 2005)

Hans-Peter Kiem, MD (2003 - 2006)

Donald B. Kohn, MD (2002 - 2005)

Jan Nolta, PhD (2002 - 2005)

Catherine Verfaillie, MD (2001 - 2004)

Christopher Walsh, MD, PhD (2003 - 2006)

# The American Society of Gene Therapy's 7th Annual Meeting final program



## Committee Roster

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### Immunology of Gene Therapy

#### Chair

Hildegund Ertl, MD (2004 - 2005)  
Michel Sadelain, MD, PhD (2003 - 2004)

#### Members

Bruce R. Blazar, MD (2003 - 2006)  
Jonathan S. Bromberg, MD, PhD (2003 - 2006)  
Karin Gaensler, MD (2003 - 2006)  
Roland W. Herzog, PhD (2004 - 2007)  
Michael Jensen, MD (2003 - 2006)  
Karin Jooss, PhD (2003 - 2006)  
Hans-Peter Kiem, MD (2003 - 2006)  
Stanley Riddell, MD (2003 - 2006)  
Isabelle Riviere, PhD (2003 - 2006)  
Michel Sadelain, MD, PhD (2003 - 2005)

### Infectious Disease

#### Chair

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John J. Rossi, PhD (2002 - 2004)

#### Members

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Bruce Bunnell, PhD (2001 - 2004)  
Boro Dropulic, PhD (2003 - 2006)  
J. Victor Garcia-Martinez, PhD (2004 - 2007)  
David Klatzmann, MD, PhD (2004 - 2007)  
Bruce L. Levine, PhD (2004 - 2007)  
Margaret A. Liu, MD (2002 - 2005)  
Vicente Planelles, PhD (2001 - 2004)  
Roger J. Pomerantz, MD, FACP (1998 - 2005)  
John J. Rossi, PhD (2002 - 2005)  
David Strayer, MD, PhD (2003 - 2006)  
Didier Trono, MD (2004 - 2007)  
John A. Zaia, MD (2002 - 2005)

### Musculo-Skeletal Disorder Gene Therapy

#### Chair

Jeffrey Chamberlain, PhD (2003 - 2005)

#### Members

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George Dickson, PhD (2004 - 2007)  
Dongsheng Duan, PhD (2004 - 2007)  
Dan Gazit, MD, PhD (2004 - 2007)  
Steven C. Ghivizzani, PhD (2004 - 2007)  
Johnny Huard, PhD (2004 - 2007)  
Laurence Kedes, MD (2001 - 2004)  
Jay R. Lieberman, MD (2003 - 2006)  
Terence A. Partridge, PhD (2004 - 2007)  
Paul D. Robbins, PhD (2003 - 2005)  
Blake Roessler, MD (2001 - 2004)  
Michael Themis, PhD (2004 - 2007)  
Jon Wolff, MD (2001 - 2007)

### Neural Disorders Gene Therapy

#### Chair

Pedro Lowenstein, MD, PhD (2003 - 2005)  
Krys Bankiewicz, MD, PhD (2004 - 2007)

#### Members

Martha C. Bohn, PhD (2001 - 2004)  
Xandra Breakefield, PhD (2004 - 2007)  
E. Antonio Chiocca, MD, PhD (2003 - 2006)  
Beverly L. Davidson, PhD (2003 - 2004)  
Nicole Deglon, PhD (2004 - 2007)  
Howard J. Federoff, MD, PhD (2001 - 2004)  
David J. Fink, MD (2002 - 2005)  
John Forsayeth, PhD (2004 - 2007)  
Sanjiv Gambhir, MD, PhD (2003 - 2006)  
Steven Goldman, MD, PhD (2001 - 2004)  
Michael Kaplitt, MD, PhD (2003 - 2006)  
George Karpati, MD (2003 - 2006)  
Daniel A. Muruve, MD (2003 - 2006)  
Edward Schuchman, PhD (2003 - 2006)  
H. David Shine, PhD (2003 - 2006)  
James B. Uney, PhD (2003 - 2006)  
John H. Wolfe, VMD, PhD (2004 - 2007)

### Nonviral Gene Transfer Vectors

#### Chair

Seng H. Cheng, PhD (2001 - 2004)  
Dexi Liu, PhD (2004 - 2007)

#### Members

Michael A. Barry, PhD (2004 - 2007)  
Michele P. Calos, PhD (2004 - 2007)  
Esther H. Chang, PhD (2003 - 2006)  
Pamela Davis, MD, PhD (2004 - 2007)  
Dieter Gruenert, PhD (2001 - 2004)  
Stephen Hart, PhD (2003 - 2006)  
Richard Heller, PhD (2002 - 2005)  
Leaf Huang, PhD (2002 - 2005)  
Alexander V. Kabanov, PhD (2003 - 2006)  
Sung Wan Kim, PhD (2002 - 2005)  
Kam Leong, PhD (2004 - 2007)  
Dexi Liu, PhD (2001 - 2004)  
Ian MacLachlan, PhD (2004 - 2007)  
Lluis M. Mir, DSc (2003 - 2006)  
Kevin G. Rice, PhD (2001 - 2004)  
Daniel Scherman, PhD (2004 - 2007)  
Sean M. Sullivan, PhD (2001 - 2004)  
Francis C. Szoka, Jr, PhD (2004 - 2007)  
Nancy Smyth Templeton, PhD (2001 - 2004)

# The American Society of Gene Therapy's 7th Annual Meeting final program

# 2004

## Committee Roster

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Jon Wolff, MD (2001 - 2004)

### Respiratory Tract Gene Therapy

*Chair*

Daniel Weiss, MD, PhD (2003 - 2005)

*Members*

Raj Batra, MD (2001 - 2006)

Bruce Bunnell, PhD (2004 - 2007)

David A. Dean, PhD (2004 - 2007)

Phillip Factor, DO (2001 - 2005)

Jim Hu, PhD (2004 - 2007)

Robert Kaner, MD (2001 - 2005)

Jay K. Kolls, MD (2001 - 2006)

Paul B. McCray, Jr, MD (2003 - 2006)

Alistair Ramsay, PhD (2003 - 2006)

Paul N. Reynolds, MD, PhD (2001 - 2004)

Guoshun Wang, DVM, PhD (2004 - 2007)

Joseph Zabner, MD (2001 - 2004)

Pamela L. Zeitlin, MD, PhD (2003 - 2006)

### Viral Gene Transfer Vectors

*Chair*

Kenneth Cornetta, MD (2004 - 2005)

Michael J. Imperiale, PhD (2002 - 2004)

*Members*

Douglas Brough, PhD (2002 - 2005)

Barry J. Byrne, MD, PhD (2004 - 2007)

Jay A. Chiorini, PhD (2003 - 2006)

Peter Colosi, PhD (2004 - 2007)

Kenneth Cornetta, MD (2002 - 2005)

Howard Federoff, MD, PhD (2004 - 2007)

Michael J. Imperiale, PhD (2002- 2005)

Nori Kasahara, MD, PhD (2003 - 2006)

Stefan Kochanek, MD (2002 - 2005)

Philip Ng, PhD (2004 - 2007)

Robin J. Parks, PhD (2003 - 2006)

Samuel D. Rabkin, PhD (2002 - 2005)

Brian P. Sorrentino, MD (2004 - 2007)

David Strayer, MD, PhD (2002 - 2005)

Christof von Kalle, MD (2003 - 2006)

Matthew Weitzman, PhD (2002 - 2005)

James M. Wilson, MD, PhD (2003 - 2006)

For a complete listing

of committee meetings

taking place at

the Annual Meeting,

see page 3

# The American Society of Gene Therapy's 7th Annual Meeting final program

2004

## ASGT 7th Annual Meeting Supporters



General Meeting Support



Partial Support for Final Program Printing

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NIH Evening Symposium

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NIH Evening Symposium



General Meeting Support

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NIH Evening Symposium

Office of Biotechnology Activities  
NIH Evening Symposium



Corporate  
Symposium



Registration Bags

VIRxSYS

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Corporate Symposium

# The American Society of Gene Therapy's 7th Annual Meeting final program

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## ASGT 7th Annual Meeting Exhibitors

Aldevron  
Althea Technologies  
Alzet® Osmotic Pumps, Durect Corporation  
amaxa GmbH  
American Society of Gene Therapy  
AMRESCO Inc.  
AppTec Laboratory Services  
Arkios BioDevelopment International  
Avanti Polar Lipids, Inc.  
BioProcess International  
BioSpherix, Ltd.  
CellGenix, GmbH  
Corning Incorporated  
Elim Biopharmaceuticals, Inc.  
Elsevier  
Genassess, Inc.  
GENEART GmbH  
HyClone  
InvivoGen  
Lark Technologies  
Mary Ann Liebert, Inc.  
MDS Pharma Services  
Mirus Corporation  
MJ Research, Inc.  
MolecularTrucks  
National Gene Vector Laboratories  
National Institutes of Health/NHLBI  
Nature Publishing Group  
Nature Technology Corporation  
Office of Biotechnology Activities, National Institutes of Health  
Olympus America Inc.  
Omnia Biologics, Inc.  
Open Biosystems  
Pall Life Sciences  
PlasmidFactory GmbH & Co. KG  
ProMetic BioSciences, Inc.  
Protech International, Inc.  
Puresyn, Inc.  
Qbiogene, Inc.  
QIAGEN Inc.  
R&D Systems, Inc.  
Sangamo BioSciences, Inc.  
Sartorius BBI Systems Inc.  
SeqWright, Inc.  
Southern Research Institute  
St. Jude Children's Research Hospital  
St. Louis Convention & Visitors Commission  
The Williamsburg BioProcessing Foundation  
Virapur, LLC  
Waisman Clinical BioManufacturing Facility  
Wiley

## Exhibit Hall Hours

### Thursday, June 3, 2004

3:00 pm – 7:30 pm

Welcome Reception begins at 4:00 pm

### Friday, June 4, 2004

3:00 pm – 7:00 pm

Networking Reception begins at 4:00 pm

### Saturday, June 5, 2004

3:00 pm – 7:00 pm

Networking Reception begins at 4:00 pm

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Reviewers

#### **Committee Chair**

Barrie J. Carter, PhD

#### **RNA Virus Vectors**

*Coordinating Reviewer*  
Paula Cannon, PhD

#### *Abstract Reviewers*

Michael Bitzer, MD, PhD  
Noriyuki Kasahara, MD, PhD  
Kyriacos Mitrophanous, PhD  
Richard Vile, PhD

#### **AAV Vectors**

*Coordinating Reviewer*  
Matthew Weitzman, PhD

#### *Abstract Reviewers*

Jay Chiorini, PhD  
Robert Kotin, PhD  
Joseph Rabinowitz, PhD  
Arun Srivastava, PhD

#### **Adenovirus Vectors**

*Coordinating Reviewer*  
Michael Imperiale, PhD

#### *Abstract Reviewers*

Andrea Amalfitano, DO, PhD  
Joseph Bruder, PhD  
Maria Croyle, PhD  
Robin Parks, PhD

#### **Other DNA Viruses**

*Coordinating Reviewer*  
Xandra Breakefield, PhD

#### *Abstract Reviewers*

David Bartlett, MD  
William Bowers, PhD  
David Fink, MD  
Richard Wade-Martins, DPhil

#### **Naked DNA Gene Transfer**

*Coordinating Reviewer*  
Dexi Liu, PhD

#### *Abstract Reviewers*

Michael Barry, PhD  
David Dean, PhD  
Todd Giorgio, PhD  
Jon Wolff, MD

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#### *Abstract Reviewers*

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Andrew Miller, PhD  
Volkmar Weissig, PhD

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Katherine Ponder, MD

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Brendan Lee, MD, PhD

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Anthony Rosenzweig, MD

#### *Abstract Reviewers*

Hunter Champion, MD, PhD  
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Roger Hajjar, MD  
Todd Rosengart, MD

#### **Neurologic**

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Pedro Lowenstein, MD, PhD

#### *Abstract Reviewers*

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Ron Mandel, PhD  
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#### *Abstract Reviewers*

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Olivier Danos, PhD  
Steven Ghivizzani, PhD  
Johnny Huard, PhD

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*Coordinating Reviewer*  
John Zaia, MD

#### *Abstract Reviewers*

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Carl June, MD  
John Rossi, PhD  
K.K. Wong, Jr, MD

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*Coordinating Reviewer*  
Helen E. Heslop, MD

#### *Abstract Reviewers*

Laura Aguilar, MD, PhD  
Catherine Bolland, MD  
Michael Jensen, MD  
Bruce Levine, PhD

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*Coordinating Reviewer*  
Jack Roth, MD

*Abstract Reviewers*  
Sunil Chada, PhD  
Bingliang Fang, MD, PhD  
David Kirn, MD  
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Svend Freytag, PhD

*Abstract Reviewers*  
Donald Buchsbaum, PhD  
William Parker, PhD  
Donna Shewach, PhD

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*Abstract Reviewers*  
David Curiel, MD, PhD  
Albert Deisseroth, MD, PhD  
C. Richter King, PhD  
Murali Ramachandra, PhD

#### **Hematologic**

*Coordinating Reviewer*  
Fabio Candotti, MD

*Abstract Reviewers*  
C. Anthony Blau, MD  
Robert Hawley, PhD  
Jan Nolta, PhD  
Michel Sadelain, MD, PhD

#### **Lung and Respiratory Disease**

*Coordinating Reviewer*  
Daniel Weiss, MD, PhD

*Abstract Reviewers*  
Raj Batra, MD  
Phillip Factor, DO  
Robert Kaner, MD  
Guoshun Wang, DVM, PhD

#### **Gene Regulation**

*Coordinating Reviewer*  
Casey Case, PhD

*Abstract Reviewers*  
John Forsayeth, PhD  
Sonya Franklin, PhD  
Brian Johnstone, PhD  
Jeffrey Nordstrom, PhD  
Matthew Porteus, MD, PhD

# The American Society of Gene Therapy's 7th Annual Meeting final program

# 2004

## ASGT Planning Committee & Group Coordinating Reviewers

David Bodine, PhD  
GMBB/NHGRI/NIH  
Hematopoiesis Section, Room 3A14  
49 Convent Drive, MSC 4442  
Bethesda, MD 20892-4442

Malcolm Brenner, MD, PhD  
Baylor College of Medicine  
6621 Fannin Street MC3-3320  
Houston, TX 77030

Barrie Carter, PhD  
Targeted Genetics Corp.  
1100 Olive Way, Suite 100  
Seattle, WA 98101

Helen Heslop, MD  
Baylor College of Medicine  
Cell & Gene Therapy Program  
1102 Bates Street, Suite 1120.07  
Houston, TX 77030

Katherine A. High, MD  
The Children's Hospital of Philadelphia  
Howard Hughes Medical Institute  
310 Abramson Research Center  
3615 Civic Center Boulevard  
Philadelphia, PA 19104-4399

Donald Kohn, MD  
Childrens Hospital Los Angeles  
Research Immunology/BMT  
4650 Sunset Boulevard, #62  
Los Angeles, CA 90027

Pedro Lowenstein, MD, PhD  
Gene Therapeutics Research Institute  
Cedars-Sinai Medical Center, Davis Building  
8700 Beverly Boulevard, Room 5093  
Los Angeles, CA 90048-1865

Robert Martuza, MD  
Massachusetts General Hospital  
Neurosurgery Department  
55 Fruit Street, WH502  
Boston, MA 02114

Ralph W. Paul, PhD  
Targeted Genetics Corp.  
1100 Olive Way, Suite 100  
Seattle, WA 98101

Katherine Ponder, MD  
Washington University School of Medicine  
Division of Hematology  
660 S. Euclid Avenue, Box 8125  
St. Louis, MO 63110

Paul Robbins, PhD  
University of Pittsburgh  
Dept. of Molecular Genetics  
W1246 Biomedical Science Tower  
Pittsburgh, PA 15261

Matthew Weitzman, PhD  
The Salk Institute  
Laboratory of Genetics  
10010 North Torrey Pines Road  
San Diego, CA 92037

**Thank You Planning Committee & Reviewers!**

# The American Society of Gene Therapy's 7th Annual Meeting final program

# 2004

## Excellence in Research Awards

For Students and Post Doctoral Fellows

### Brian Beard – Post Doctoral Fellow

Molecular Long-Term Follow up in Non-Human Primates That Received Retrovirally Transduced Stem Cells: Despite Proto-Oncogene Insertion No Leukemia or Hematopoietic Abnormalities

### Alessandra Biffi – Post Doctoral Fellow

Ex Vivo Gene Therapy Provides a Unique Therapeutic Benefit and Corrects Metachromatic (MLD) and Globoid (GLD) Leukodystrophy in Mouse Models

### Martijn Brugman – Student

Insertional Leukemogenesis After Retroviral Gene Transfer into Murine Hematopoietic Stem Cells

### Hiroshi Nakano – Student

IL-21 and IL-15 Gene Therapy Induces Powerful Antitumor Effect Against Head and Neck Squamous Cell Carcinoma

### \*Geetha Puthenveetil – Post Doctoral Fellow

Successful Correction of Human Beta Thalassemia Major Using a Self-Inactivating Lentiviral Vector

### Rahul Srinivasan – Student

HSV Based Genetic Selection System for Identification of Pain Signaling Regulators

*The Excellence in Research Awards were made partially possible through grant support from Fanconi Anemia Research Fund, Inc. and Introgen Therapeutics.*

*\* Fanconi Anemia Research Fund Recipient*

## Travel Grant Recipients

Majed Aljamali

Kenneth Barton

Brian Beard

Alessandra Biffi

Katherine Bowman

Martijn Brugman

Boris Calmels

Pierre Chenuaud

Caroline Cole

Kerry Cresawn

Emmanuel Dauty

Douglas Dylla

Anja Ehrhardt

Maaike Everts

Paul Gregorevic

Josh Grieger

Dirk Grimm

Sundeep Keswani

Gary Kobinger

Beat Kramer

Florian Kreppel

Yasuo Kunugiza

Ming-Jie Li

Joke Lievens

Aaron Logan

Todd Miller

Sara Muraro

Hiroshi Nakano

Katsutoshi Nakayama

Eric Poeschla

Geetha Puthenveetil

Colin Ross

Hiroki Saitoh

Rahul Srinivasan

John Stagg

Ennio Tasciotti

Maria Vera Ugalde

Lingfei Xu

Ziying Yan

Xiaojing Ye

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

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### Faculty List

#### **Patrick Aebscher, MD**

Institute of Neuroscience  
Lausanne, Switzerland

#### **Estuardo Aguilar-Cordova, PhD**

Advantagene  
Waban, Massachusetts

#### **Gustavo D. Aguirre, VMD, PhD**

James A. Baker Institute for  
Animal Health  
Ithaca, New York

#### **Steven M. Albelda, MD**

University of Pennsylvania  
Philadelphia, Pennsylvania

#### **Tomas S. Aleman, MD**

University of Pennsylvania School of  
Medicine Scheie Eye Institute  
Philadelphia, Pennsylvania

#### **Andrea Amalfitano, DO, PhD**

Duke University Medical Center  
Durham, North Carolina

#### **W. French Anderson, MD**

University of Southern California  
Keck School of Medicine  
Los Angeles, California

#### **Rosemarie E. Aurigemma, PhD**

National Cancer Institute - Frederick  
Frederick, Maryland

#### **Andrew H. Baker, PhD**

University of Glasgow  
Glasgow, Scotland

#### **Michael A. Barry, PhD**

Baylor College of Medicine  
Houston, Texas

#### **David L. Bartlett, MD**

University of Pittsburgh Medical  
Center Cancer Pavilion  
Pittsburgh, Pennsylvania

#### **Jeffrey S. Bartlett, PhD**

Children's Research Institute  
Columbus, Ohio

#### **Raj Batra, MD**

UCLA/The West LA Veterans  
Administration Medical Center  
Los Angeles, California

#### **Christopher Baum, MD**

Medizinische Hochschule Hannover  
Hannover, Germany

#### **Jean Bennett, MD, PhD**

University of Pennsylvania  
Scheie Eye Institute  
Philadelphia, Pennsylvania

#### **Terry R. Bishop, PhD**

National Institutes of Health  
Bethesda, Maryland

#### **Michael Bitzer, MD, PhD**

Medical University Clinic  
Tübingen, Germany

#### **R. Michael Blaese, MD**

Fund Inherited Disease Research  
New Hope, Pennsylvania

#### **C. Anthony Blau, MD**

University of Washington  
Seattle, Washington

#### **Bruce R. Blazar, MD**

University of Minnesota Hospital  
Minneapolis, Minnesota

#### **Daniel Boden, MD**

Aaron Diamond AIDS  
Research Center  
New York, New York

#### **Catherine M. Bolland, MD**

Baylor College of Medicine  
Houston, Texas

#### **Claudio Bordignon, MD**

Istituto Scientifico H. San Raffaele  
Milano, Italy

#### **William J. Bowers, PhD**

University of Rochester  
School of Medicine  
Rochester, New York

#### **Xandra Breakefield, PhD**

Massachusetts General Hospital  
Charlestown, Massachusetts

#### **Malcolm Brenner, MD, PhD**

Baylor College of Medicine  
Houston, Texas

#### **Jonathan S. Bromberg, MD, PhD**

Mount Sinai School of Medicine  
New York, New York

#### **Joseph T. Bruder, PhD**

GenVec, Inc.  
Gaithersburg, Maryland

#### **Donald Buchsbaum, PhD**

University of Alabama at Birmingham  
Birmingham, Alabama

#### **Haim Burstein, PhD**

Targeted Genetic Corporation  
Seattle, Washington

#### **Frederic D. Bushman, PhD**

University of Pennsylvania  
School of Medicine  
Philadelphia, Pennsylvania

#### **Barry J. Byrne, MD, PhD**

University of Florida  
College of Medicine  
Gainesville, Florida

#### **Michele P. Calos, PhD**

Stanford University School of Medicine  
Stanford, California

#### **Paula M. Cannon, PhD**

Childrens Hospital Los Angeles  
Los Angeles, California

#### **Casey C. Case, PhD**

Sangamo BioSciences, Inc.  
Richmond, California

#### **Maria Castro, PhD**

Gene Therapeutics Research Institute  
Los Angeles, California

#### **Joy Cavagnaro, PhD, DABT, RAC**

Access BIO  
Leesburg, Virginia

#### **Sunil Chada, PhD**

Introgen Therapeutics, Inc.  
Houston, Texas

#### **Jeffrey Chamberlain, PhD**

University of Washington  
School of Medicine  
Seattle, Washington

#### **Stephen M.W. Chang, PhD**

Poway, California

#### **Theresa Chen, PhD**

Food and Drug Administration  
Rockville, Maryland

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

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### Faculty List

#### Seng H. Cheng, PhD

Genzyme Corporation  
Framingham, Massachusetts

#### E. Antonio Chiocca, MD, PhD

The Ohio State University  
Medical Center  
Columbus, Ohio

#### Jay A. Chiorini, PhD

National Institutes of Health  
Bethesda, Maryland

#### Narendra Chirmule, PhD

Merck & Co., Inc.  
Wayne, Pennsylvania

#### Odile Cohen-Haguener, MD, PhD

Euregenethy  
Cachan, France

#### Laurence JN Cooper, MD, PhD

City of Hope  
National Medical Center  
Duarte, California

#### Mark J. Cooper, MD

Copernicus Therapeutics, Inc.  
Cleveland, Ohio

#### Larry A. Couture, PhD

Beckman Research Institute of  
City of Hope  
Duarte, California

#### Maria A. Croyle, PhD

The University of Texas at Austin  
College of Pharmacy  
Austin, Texas

#### Ronald G. Crystal, MD

Weill Medical College of  
Cornell University  
New York, New York

#### David T. Curiel, MD, PhD

University of Alabama at  
Birmingham Gene Therapy Center  
Birmingham, Alabama

#### George Q. Daley, MD, PhD

Children's Hospital  
Boston, Massachusetts

#### Olivier Danos, PhD

Genethon  
Evry, France

#### Beverly L. Davidson, PhD

University of Iowa  
Iowa City, Iowa

#### Pamela Davis, MD, PhD

Case Western Reserve University  
Cleveland, Ohio

#### David A. Dean, PhD

Northwestern University  
Medical School  
Chicago, Illinois

#### Albert B. Deisseroth, MD, PhD

Sidney Kimmel Cancer Center  
San Diego, California

#### Giovanni Di Pasquale, PhD

National Institute of Dental and  
Craniofacial Research  
Bethesda, Maryland

#### David A. Dichek, MD

University of Washington  
Seattle, Washington

#### John E. Dick, PhD

Toronto General Research Institute/  
University Health Network  
Toronto, Canada

#### Peter Dirks, MD, PhD, FRCSC

Hospital for Sick Children  
Toronto, Canada

#### J. Kevin Donahue, MD

Johns Hopkins University  
Baltimore, Maryland

#### Boro Dropulic, PhD

VIRxSYS Corporation  
Gaithersburg, Maryland

#### Steven Dubinett, MD

University of California Los Angeles  
Los Angeles, California

#### David W. Emery, PhD

University of Washington  
Seattle, Washington

#### John F. Engelhardt, PhD

University of Iowa  
College of Medicine  
Iowa City, Iowa

#### Phillip Factor, DO

New York Presbyterian Hospital  
New York, New York

#### Howard J. Federoff, MD, PhD

University of Rochester  
Rochester, New York

#### Terence R. Flotte, MD

University of Florida  
Gainesville, Florida

#### John Forsayeth, PhD

Avigen, Inc.  
Alameda, California

#### Sonya J. Franklin, PhD

University of Iowa  
Iowa City, Iowa

#### Svend O. Freytag, PhD

Henry Ford Health System  
Detroit, Michigan

#### Theodore Friedmann, MD

University of California San Diego  
School of Medicine  
La Jolla, California

#### Juan Fueyo, MD

M.D. Anderson Cancer Center  
Houston, Texas

#### Guangping Gao, PhD

University of Pennsylvania  
Philadelphia, Pennsylvania

#### Steven C. Ghivizzani, PhD

Brigham and Women's Hospital  
Boston, Massachusetts

#### Mauro Giacca, MD, PhD

International Centre for Genetic  
Engineering and Biotechnology  
Trieste, Italy

#### Eli Gilboa, PhD

Duke University Medical Center  
Durham, North Carolina

#### Frank J. Giordano, MD

Yale School of Medicine  
New Haven, Connecticut

#### Todd Giorgio, PhD

Vanderbilt University  
Nashville, Tennessee

#### Joel N. Glasgow, PhD

Vector Logics  
Birmingham, Alabama

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### Faculty List

#### **Joseph C. Glorioso, PhD**

University of Pittsburgh  
School of Medicine  
Pittsburgh, Pennsylvania

#### **Margaret A. Goodell, PhD**

Baylor College of Medicine  
Houston, Texas

#### **Stephen Gottschalk, MD**

Baylor College of Medicine  
Houston, Texas

#### **Philip D. Gregory, PhD**

Sangamo BioSciences, Inc.  
Richmond, California

#### **Dieter Gruenert, PhD**

California Pacific Medical Center  
Research Institute  
San Francisco, California

#### **Neil R. Hackett, PhD**

Weill Medical College of Cornell  
University  
New York, New York

#### **Roger J. Hajjar, MD**

Massachusetts General Hospital  
Charlestown, Massachusetts

#### **Michael Hallek, MD**

University Hospital Cologne  
Cologne, Germany

#### **Cary Harding, MD**

Oregon Health Sciences University  
Portland, Oregon

#### **Thomas Harding, PhD**

Cell Genesys, Inc.  
South San Francisco, California

#### **Reuben S. Harris, PhD**

University of Minnesota  
Minneapolis, Minnesota

#### **Ben-Gary Harvey, MD**

Weill Medical College of Cornell  
University  
New York, New York

#### **Mark Haskins, VMD, PhD**

University of Pennsylvania  
Philadelphia, Pennsylvania

#### **John G. Hay, MD**

New York University  
School of Medicine  
New York, New York

#### **Akseli E. Hemminki, MD, PhD**

University of Helsinki  
Helsinki, Finland

#### **Roland W. Herzog, PhD**

The Children's Hospital of Philadelphia  
Philadelphia, Pennsylvania

#### **Katherine A. High, MD**

The Children's Hospital of Philadelphia  
Philadelphia, Pennsylvania  
Howard Hughes Medical Institute  
Chevy Chase, Maryland

#### **Thomas J. Hope, PhD**

University of Illinois at Chicago  
College of Medicine  
Chicago, Illinois

#### **Johnny Huard, PhD**

Children's Hospital of Pittsburgh  
Pittsburgh, Pennsylvania

#### **Beth Hutchins, PhD**

Canji, Inc.  
San Diego, California

#### **Stephen Hyde, PhD**

University of Oxford  
Oxford, United Kingdom

#### **Michael J. Imperiale, PhD**

University of Michigan  
Ann Arbor, Michigan

#### **Andreas H. Jacobs, MD**

Max-Planck-Institute for  
Neurological Research  
Cologne, Germany

#### **Michael Jensen, MD**

City of Hope National  
Medical Center  
Duarte, California

#### **Philip R. Johnson, MD**

Children's Research Institute  
Columbus, Ohio

#### **Brian Johnstone, PhD**

Indiana Center for Vascular  
Biology and Medicine  
Indianapolis, Indiana

#### **Douglas J. Jolly, PhD**

Advantagene Inc.  
Encinitas, California

#### **Karin Jooss, PhD**

Cell Genesys, Inc.  
South San Francisco, California

#### **Craig T. Jordan, PhD**

University of Rochester  
School of Medicine  
Rochester, New York

#### **Carl H. June, MD**

University of Pennsylvania  
Philadelphia, Pennsylvania

#### **Robert Kaner, MD**

Weill Medical College of Cornell  
University  
New York, New York

#### **Michael G. Katze, PhD**

University of Washington  
Seattle, Washington

#### **Mark A. Kay, MD, PhD**

Stanford University School of Medicine  
Stanford, California

#### **Gordon Keller, PhD**

Mount Sinai School of Medicine  
New York, New York

#### **Hans-Peter Kiem, MD**

Fred Hutchinson Cancer  
Research Center  
Seattle, Washington

#### **David H. Kirn, MD**

JENNEREX Pharmaceuticals  
Mill Valley, California

#### **David Klatzmann, MD, PhD**

Cervi-Hopital Litié  
Paris, France

#### **Aaron Klug, OM, FRS**

Cambridge University  
Cambridge, United Kingdom

#### **Richard A. Knazek, MD**

National Center for Research  
Resources  
Bethesda, Maryland

# The American Society of Gene Therapy's 7th Annual Meeting

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### Faculty List

#### **Walter J. Koch, PhD**

Thomas Jefferson University  
Philadelphia, Pennsylvania

#### **Stefan Kochanek, MD**

University of Ulm  
Ulm, Germany

#### **Donald B. Kohn, MD**

Children's Hospital Los Angeles  
Los Angeles, California

#### **Joe Kornegay, DVM, PhD**

University of Missouri  
Columbia, Missouri

#### **Robert J. Korst, MD**

Weill Medical College of  
Cornell University  
New York, New York

#### **Robert M. Kotin, PhD**

National Heart, Lung and  
Blood Institute  
Bethesda, Maryland

#### **Art Krieg, MD**

Coley Pharmaceutical Group  
Wellesley, Massachusetts

#### **Long P. Le, PhD**

University of Alabama at Birmingham  
Birmingham, Alabama

#### **Brendan Lee, MD, PhD**

Baylor College of Medicine  
Houston, Texas  
Howard Hughes Medical Institute  
Chevy Chase, Maryland

#### **Kam W. Leong, PhD**

Johns Hopkins School of Medicine  
Baltimore, Maryland

#### **Philip L. Leopold, PhD**

Weill Medical College of  
Cornell University  
New York, New York

#### **Fong-Qi (Frank) Liang, MD, PhD**

Retina Foundation of the Southwest  
Dallas, Texas

#### **Judy Lieberman, MD, PhD**

The Center for Blood Research  
Institute for Biomedical Research  
Harvard Medical School  
Boston, Massachusetts

#### **Margaret A. Liu, MD**

Transgene  
Lafayette, California

#### **Xiaoming Liu, PhD**

The University of Iowa  
Iowa City, Iowa

#### **Robert M. Lorence, MD, PhD**

Wellstat Biologics Corporation  
Gaithersburg, Maryland

#### **Douglas Losordo, MD**

St. Elizabeth's Medical Center  
Boston, Massachusetts

#### **Pedro Lowenstein, MD, PhD**

Gene Therapeutics Research Institute  
Los Angeles, California

#### **Lucio Luzzatto, MD, PhD**

Istituto Nazionale Per La Ricerca  
Sul Cancro  
Genova, Italy

#### **Ian MacLachlan, PhD**

Protiva Biotherapeutics Inc.  
Burnaby, Canada

#### **Punam Malik, MD**

Children's Hospital Los Angeles  
Los Angeles, California

#### **Ronald Mandel, PhD**

Gene Therapy Center  
Gainesville, Florida

#### **Robert L. Martuza, MD**

Massachusetts General Hospital  
Boston, Massachusetts

#### **Lorraine Matheson**

National Gene Vector Laboratories  
Indianapolis, Indiana

#### **Paul B. McCray, Jr, MD**

University of Iowa College of Medicine  
Iowa City, Iowa

#### **Maritza C. McIntyre, PhD**

Food and Drug Administration  
Rockville, Maryland

#### **Michael McManus, PhD**

Massachusetts Institute of Technology  
Cambridge, Massachusetts

#### **Carol H. Miao, PhD**

University of Washington  
Seattle, Washington

#### **Andrew D. Miller, PhD**

Imperial College Genetic  
Therapies Centre  
London, United Kingdom

#### **Daniel G. Miller, MD, PhD**

University of Washington  
Seattle, Washington

#### **Lluís M. Mir, DSc**

Centre National de la Recherche  
Scientifique - Institute Gustave-  
Roussy  
Villejuif, France

#### **Kyriacos A. Mitrophanous, PhD**

Oxford Biomedica  
Oxford, United Kingdom

#### **James J. Mulé, PhD**

H. Lee Moffitt Cancer Center &  
Research Institute  
Tampa, Florida

#### **Daniel A. Muruve, MD**

University of Calgary  
Calgary, Canada

#### **Josephine Nalbantoglu, PhD**

McGill University  
Montreal, Canada

#### **Luigi Naldini, MD, PhD**

San Raffaele Telethon Institute  
for Gene Therapy  
Milan, Italy

#### **John J. Nemunaitis, MD**

US Oncology  
Dallas, Texas

#### **Timothy C. Nichols, MD**

University of North Carolina  
at Chapel Hill  
Chapel Hill, North Carolina

#### **Alan J. Nixon, BVSc, MS**

Cornell University  
Ithaca, New York

#### **Philip D. Noguchi, MD**

Food and Drug Administration  
Rockville, Maryland

#### **Jan Nolta, PhD**

Washington University  
School of Medicine  
St. Louis, Missouri

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Faculty List

#### **Jeffrey L. Nordstrom, PhD**

College Station, Texas

#### **Wanda O'Neal, PhD**

University of North Carolina at Chapel Hill  
Chapel Hill, North Carolina

#### **Luis Ortiz, MD**

University of Pittsburgh  
Pittsburgh, Pennsylvania

#### **Trevor Owens, PhD**

Montreal Neurological Institute  
Montreal, Canada

#### **John G. Page, PhD, DABT**

Southern Research Institute  
Birmingham, Alabama

#### **Thalia Papayannopoulou, MD**

University of Washington School of Medicine  
Seattle, Washington

#### **Robin J. Parks, PhD**

Ottawa Health Research Institute  
Ottawa, Canada

#### **Terence A. Partridge, PhD**

Imperial College School of Medicine  
London, United Kingdom

#### **Ralph W. Paul, PhD**

Targeted Genetics Corporation  
Seattle, Washington

#### **Paula Pitha-Rowe, PhD**

Johns Hopkins School of Medicine  
Baltimore, Maryland

#### **Katherine Ponder, MD**

Washington University School of Medicine  
Saint Louis, Missouri

#### **Matthew H. Porteus, MD, PhD**

University of Texas Southwestern Medical School  
Dallas, Texas

#### **Peter Quesenberry, MD**

Roger Williams Medical Center  
Providence, Rhode Island

#### **Joseph Rabinowitz, PhD**

University of North Carolina at Chapel Hill  
Chapel Hill, North Carolina

#### **Murali Ramachandra, PhD**

Canji, Incorporated  
San Diego, California

#### **Glenn Randall, PhD**

Rockefeller University  
New York, New York

#### **Eyal Raz, MD**

University of California San Diego  
La Jolla, California

#### **Theresa M. Reineke, PhD**

The University of Cincinnati  
Cincinnati, Ohio

#### **Susan D. Reynolds, PhD**

University of Pittsburgh  
Pittsburgh, Pennsylvania

#### **Stanley Riddell, MD**

Fred Hutchinson Cancer Research Center  
Seattle, Washington

#### **Victor M. Rivera, PhD**

ARIAD Pharmaceuticals  
Cambridge, Massachusetts

#### **Isabelle Riviere, PhD**

Memorial Sloan Kettering Cancer Center  
New York, New York

#### **Paul D. Robbins, PhD**

University of Pittsburgh  
Pittsburgh, Pennsylvania

#### **Harriet L. Robinson, PhD**

Yerkes Regional Primate Research Center  
Atlanta, Georgia

#### **Alain Rolland, PharmD, PhD**

Vical Inc.  
San Diego, California

#### **Dennis R. Roop, PhD**

Baylor College of Medicine  
Houston, Texas

#### **Daniel Rosenblum, MD**

Food and Drug Administration  
Rockville, Maryland

#### **Todd K. Rosengart, MD**

Northwestern University Medical School  
Evanston, Illinois

#### **Fabio Rossi, MD, PhD**

University of British Columbia  
Vancouver, Canada

#### **John J. Rossi, PhD**

Beckman Research Institute  
City of Hope  
Duarte, California

#### **David B. Rozema, PhD**

Mirus Corporation  
Madison, Wisconsin

#### **David W. Russell, MD, PhD**

University of Washington  
Seattle, Washington

#### **Stephen J. Russell, MD, PhD**

Mayo Clinic  
Rochester, Minnesota

#### **Michel Sadelain, MD, PhD**

Memorial Sloan-Kettering Cancer Center  
New York, New York

#### **Daniel R. Salomon, MD**

The Scripps Research Institute  
La Jolla, California

#### **David V. Schaffer, PhD**

University of California Berkeley  
Berkeley, California

#### **Connie Schmaljohn, PhD**

US Army Medical Research Institute of Infectious Diseases  
Frederick, Maryland

#### **Allan Shipp**

National Institutes of Health  
Bethesda, Maryland

#### **Stephanie Simek, PhD**

Food and Drug Administration  
Rockville, Maryland

#### **Sonia Skarlatos, PhD**

National Institutes of Health  
Bethesda, Maryland

#### **Richard O. Snyder, PhD**

University of Florida College of Medicine  
Gainesville, Florida

#### **Robert E. Sobol, MD**

Introgen Therapeutics, Inc.  
San Diego, California

# The American Society of Gene Therapy's 7th Annual Meeting

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# 2004

### Faculty List

#### **Jeffrey L. Spees, PhD**

Tulane University  
Health Sciences Center  
New Orleans, Louisiana

#### **Patrick Squiban, MD**

Transgene Medical Affairs  
Strasbourg, France

#### **Arun Srivastava, PhD**

Indiana University  
School of Medicine  
Indianapolis, Indiana

#### **George Stamatoyannopoulos, MD**

University of Washington  
Seattle, Washington

#### **Daniel H. Sterman, MD**

University of Pennsylvania  
Medical Center  
Philadelphia, Pennsylvania

#### **David Strayer, MD, PhD**

Jefferson Medical College  
Philadelphia, Pennsylvania

#### **Lorenz Studer, MD**

Memorial Sloan-Kettering  
Cancer Center  
New York, New York

#### **Sean M. Sullivan, PhD**

University of Florida  
College of Pharmacy  
Gainesville, Florida

#### **Geoff Symonds, PhD**

Johnson & Johnson Research  
Sydney, Australia

#### **Francis C. Szoka, Jr, PhD**

University of California San Francisco  
San Francisco, California

#### **Michael Themis, PhD**

Imperial College  
London, United Kingdom

#### **Didier Trono, MD**

University of Geneva  
Geneva, Switzerland

#### **Jeffrey Ulmer, PhD**

Chiron Vaccines  
Emeryville, California

#### **Catherine Verfaillie, MD**

University of Minnesota  
Minneapolis, Minnesota

#### **Inder Verma, PhD**

The Salk Institute  
La Jolla, California

#### **Richard G. Vile, PhD**

Mayo Clinic  
Rochester, Minnesota

#### **Christof von Kalle, MD**

Cincinnati Children's Hospital  
Research Foundation  
Cincinnati, Ohio

#### **Samuel C. Wadsworth, PhD**

Genzyme Corporation  
Framingham, Massachusetts

#### **Christopher Walsh, MD, PhD**

Mount Sinai School of Medicine  
New York, New York

#### **Guoshun Wang, DVM, PhD**

Louisiana State University Health  
Sciences Center  
New Orleans, Louisiana

#### **Kenneth H. Warrington, Jr, PhD**

University of Florida  
Gainesville, Florida

#### **Deborah J. Watson, PhD**

University of Pennsylvania  
Philadelphia, Pennsylvania

#### **Daniel Weiss, MD, PhD**

University of Vermont Health Science  
Research Facility  
Burlington, Vermont

#### **Volkmar Weissig, PhD**

Northeastern University  
Boston, Massachusetts

#### **Matthew Weitzman, PhD**

The Salk Institute  
San Diego, California

#### **Jennifer L. West, PhD**

Rice University  
Houston, Texas

#### **Katie Whartenby, PhD**

Johns Hopkins University School  
of Medicine  
Baltimore, Maryland

#### **James M. Wilson, MD, PhD**

University of Pennsylvania  
Philadelphia, Pennsylvania

#### **William Wold, PhD**

St. Louis University  
School of Medicine  
St. Louis, Missouri

#### **Jon Wolff, MD**

University of Wisconsin Madison  
Madison, Wisconsin

#### **Savio L.C. Woo, PhD**

Mount Sinai School of Medicine  
New York, New York

#### **Xiao Xiao, PhD**

University of Pittsburgh  
Pittsburgh, Pennsylvania

#### **Yuhong Xu, PhD**

Shanghai Jiaotong University  
Shanghai, Peoples Republic of China

#### **Vijay K. Yechoor, MD**

Baylor College of Medicine  
Houston, Texas

#### **Seppo Yla-Herttula, MD, PhD**

University of Kuopio  
Kuopio, Finland

#### **Mervin Yoder, MD**

Cancer Research Institute  
Indiana University  
School of Medicine  
Indianapolis, Indiana

#### **DC Yu, PhD**

Cell Genesys, Incorporated  
South San Francisco, California

#### **Joseph Zabner, MD**

University of Iowa  
Iowa City, Iowa

#### **Kurt R. Zinn, DVM, PhD**

University of Alabama at Birmingham  
Birmingham, Alabama

# The American Society of Gene Therapy's 7th Annual Meeting

## final program



### General Meeting Information

#### American Society of Gene Therapy

The American Society of Gene Therapy (ASGT) is the largest medical professional organization representing researchers and scientists dedicated to discovering new gene therapies. ASGT was established in 1996, and has approximately 2,500 members from around the world. ASGT is committed to:

- promoting and fostering the exchange and dissemination of information and ideas related to gene therapy;
- encouraging excellence in research in the field of gene therapy; and
- promoting professional and public education in all areas of gene therapy.

#### Needs

Clinical gene transfer has become increasingly complex due to ongoing developments in the fields of gene therapy itself, together with bioethics, research integrity, and financial conflicts, as well as new federal mandates, regulations, and guidelines. This meeting will provide an educational forum for researchers, scientists, and clinical investigators to expand their knowledge about the broad developments in these fields.

#### Goals

The goals of ASGT are to engage in scientific and educational activities in order to promote and foster the exchange of information and ideas relating to gene therapy, to encourage the general field of research involving gene therapy, and to promote professional and public education in all areas of gene therapy. The ASGT 7<sup>th</sup> Annual Meeting will serve as a comprehensive forum to achieve these goals.

#### Educational Objectives

At the conclusion of the activity, the participant should be able to:

- Discuss key aspects of the current laboratory and/or clinical research in gene therapy.
- Demonstrate knowledge of methods being used by gene therapy researchers and practitioners.
- Describe one or more stimulating new areas of inquiry in the area of gene therapy.
- Demonstrate familiarity with federal regulations and guidelines for clinical gene transfer studies.

#### Educational Methods and Materials

Lectures, Case Presentations, Panel Discussions, Question and Answer Sessions, Workshops, Audio/Visual Presentations, Abstracts, Posters, Executive Summary

#### Target Audience

The target audience includes basic science and translational researchers, clinical investigators, physicians, employees of federal government and regulatory agencies, postdoctoral fellows, graduate students, and other healthcare professionals with an interest in the latest advancements in the field of gene therapy.

#### Evaluation Method

Evaluation by questionnaire will address program content, presentation, and possible bias.

#### Evaluations

Please take time to complete the evaluation forms distributed at each session you attend. Your input and comments are essential in planning future educational sessions. When completed, evaluations can be returned to the ushers assigned to your session, any ASGT staff person, or dropped in the evaluation collection boxes located throughout the Convention Center.

#### Accreditation/Credit Designation

This activity has been planned and implemented in accordance with the Essential Areas and policies of the Accreditation Council for Continuing Medical Education through the joint sponsorship of Baylor College of Medicine and the American Society of Gene Therapy. Baylor College of Medicine is accredited by the ACCME to provide continuing medical education for physicians.

Baylor College of Medicine designates this educational activity for a maximum of 30.25 category 1 credits toward the AMA Physician's Recognition Award. Each physician should claim only those credits that he/she actually spent in the activity.

#### Disclosure

It is the policy of Baylor College of Medicine (Baylor) that the provider and faculty/presenters disclose any significant financial or other relationships with commercial companies whose products may be discussed in the activity, or the commercial supporters, if any. Baylor also requires that faculty disclose any unlabeled use or investigational use (not yet approved for any purpose) of pharmaceutical and medical device products. Specific disclosure will be made to the participants prior to the educational activity.

Faculty, topics, program schedule, and credit are subject to change.

Audio or videotaping is prohibited without written permission from the Program Director and the Office of Continuing Medical Education, Baylor College of Medicine, Houston, Texas.

# The American Society of Gene Therapy's 7th Annual Meeting

## final program



### General Meeting Information (continued)

#### CME Certificates

CME Credit Certificates will be available at the registration desk for participants requiring CME credit for this meeting. Participants must complete the form onsite or mail the form to Baylor College of Medicine, Office of Continuing Medical Education, within five business days.

#### CME Sign In

All participants requiring CME credit must sign in each day they are in attendance at the ASGT 7<sup>th</sup> Annual Meeting. The sign in area is adjacent to the registration desk. A CME Credit Certificate will not be available unless you sign in each day of the meeting.

#### Abstract Volume

All abstracts accepted for presentation at the ASGT 7<sup>th</sup> Annual Meeting have been published in the May supplement of *Molecular Therapy*. Each attendee will receive one copy of the supplement along with other registration materials.

#### Admission to Sessions

Official name badges will be required for admission to all ASGT sessions. All Annual Meeting attendees receive a name badge with their registration bag. Name badges should be worn at all times inside the convention center, as badges will be used to control access to sessions and activities. Attendees are cautioned against wearing their name badges while away from the convention center as badges may draw unwanted attention to your status as visitors to Minneapolis.

#### Admission by Ticket Only

The Meet-the-Investigator sessions require tickets for admission. Tickets for these sessions are not included in the meeting's regular registration fees. You must have a ticket to gain entrance into a session, which will be collected at the door by ushers. Tickets are still available to some sessions, and may be obtained at the Registration Desk. All tickets are \$20.

#### Business Centers

Business centers are available for attendees at the Minneapolis Convention Center and the Hyatt Regency Minneapolis. Please stop by either of the centers for a complete list of fees, services and hours.

#### Business Meeting/Election Results

See page 3 for Business Meeting information.

#### Committee Meetings

See page 3 for Committee Meeting information.

#### Guest Attendance

ASGT asks registered attendees to refrain from taking children, spouses, or guests to any educational session or functions offered at the 7<sup>th</sup> Annual Meeting.

#### Cell Phones

Please turn your cell phone off while attending sessions.

#### No Smoking

Smoking is prohibited at all 7<sup>th</sup> Annual Meeting sessions and events.

#### Job Bank

All job openings posted as of May 19, 2004, on the ASGT Job Bank have been included in the Jobs Available Booklet found in the registration bags. Contact information is included in the booklet.

Job postings for open positions and resumes may be posted on the Job Posting Area boards in the registration area. You are responsible for making copies of items you wish to post. Please do not post job openings or resumes on the ASGT Message Board; they will be removed.

#### Internet Kiosk Policy

Internet access will be available to meeting attendees in two locations. One kiosk is located near the Registration Desk in Lobby D and the second is near the 211 meeting rooms on Level Two. Please limit your Internet use to 15 minutes at a time so that other attendees can access the Internet as well.

#### Special Accessibility Needs

We encourage participation by all individuals. If you require special accommodations and did not previously contact the ASGT Executive Office, please go to the Registration Desk to submit your request. The ASGT staff will be happy to assist you with your specific needs.

# The American Society of Gene Therapy's 7th Annual Meeting final program



## General Meeting Information (continued)

### Exhibits

Exhibits will be located in Hall D (Level One) of the Minneapolis Convention Center. Please allow adequate time in your daily schedule to visit the exhibits. Please take the time to speak with representatives of companies that provide services or market products directly related to your professional needs. Per ASGT policy, promotional materials will not be permitted in the meeting halls, registration or reception areas or in any location of the Minneapolis Convention Center outside the rented exhibit space.

A Welcome Reception will be held in the Exhibit Hall on Thursday, June 3, and Networking Receptions in the Exhibit Hall on Friday, June 4, and Saturday, June 5. Receptions will begin at 4:00 pm daily.

### Exhibit Hall Hours

Thursday, June 3	3:00 pm – 7:30 pm
Friday, June 4	3:00 pm – 7:00 pm
Saturday, June 5	3:00 pm – 7:00 pm

### Lead Retrieval

A lead retrieval system has been made available to all exhibitors of the ASGT 7<sup>th</sup> Annual Meeting. Exhibitors may ask to scan attendee name badges with a hand held scanner in order to obtain attendee contact information.

### Posters

Abstract Posters will be on display in Hall D.

#### Thursday, June 3

##### Poster Session I – Abstracts

10:00 am – Noon	Poster Setup by Authors
Noon – 7:30 pm	Poster Viewing (authors present from 4:00 pm – 7:30 pm)
3:00 pm – 7:30 pm	Exhibit Hall Open
4:00 pm – 7:30 pm	Welcome Reception
7:30 pm – 8:00 pm	Authors Remove Posters

#### Friday, June 4

##### Poster Session II – Abstracts

7:00 am – Noon	Poster Setup by Authors
Noon – 7:00 pm	Poster Viewing (authors present from 4:00 pm – 7:00 pm)
3:00 pm – 7:00 pm	Exhibit Hall Open
4:00 pm – 7:00 pm	Networking Reception
7:00 pm – 8:00 pm	Authors Remove Posters

#### Saturday, June 5

##### Poster Session III – Abstracts

7:00 am – Noon	Poster Setup by Authors
Noon – 7:00 pm	Poster Viewing (authors present from 4:00 pm – 7:00 pm)
3:00 pm – 7:00 pm	Exhibit Hall Open
4:00 pm – 7:00 pm	Networking Reception
7:00 pm – 8:00 pm	Authors Remove Posters

### Press Room

The Press Room is located in the Minneapolis Convention Center, Room 204A.

Members of the working media may register without charge for the 7<sup>th</sup> Annual Meeting in the Press Room. Press conferences on previously selected newsworthy abstracts, along with other scientific sessions, will be conducted throughout the meeting. Interview space, computers, telephones, and fax services are available for the convenience of media representatives covering the meeting. Press must register, provide credentials, and wear their press badge for admittance to ASGT sessions.

### Press Room Hours

Wednesday, June 2	3:00 pm – 6:00 pm
Thursday, June 3	7:45 am – 5:00 pm
Friday, June 4	7:45 am – 5:00 pm
Saturday, June 5	7:45 am – 5:00 pm

# The American Society of Gene Therapy's 7th Annual Meeting final program

# 2004

## General Meeting Information (continued)

### Registration Desk

Name badges, final programs, and abstract supplements will be distributed at the registration desk located in Lobby D of the Minneapolis Convention Center.

### Registration Desk Hours

Wednesday, June 2	Noon – 8:00 pm
Thursday, June 3	7:00 am – 5:00 pm
Friday, June 4	7:30 am – 5:00 pm
Saturday, June 5	7:30 am – 5:00 pm
Sunday, June 6	7:30 am – Noon

### Restaurant Information Desk

Minneapolis offers a variety of restaurants from casual to elegant, with prices and cuisine to meet all tastes and budgets. The Information Desk is staffed by the Greater Minneapolis Convention & Visitors Association to help you make the right dining choice. Staff will have sample menus from more than 150 area restaurants and will be able to assist you in making your reservations. The Restaurant Information Desk is located in Lobby D of the Minneapolis Convention Center.

### Speaker Ready Room

The Speaker Ready Room is located in Room 103F (Level One) of the Minneapolis Convention Center.

All faculty must drop off their slides or presentations in the Speaker Ready Room the day before their session or at least four hours prior to their presentations. Equipment is available for faculty to review their materials. Audiovisual personnel will be available for assistance. Please mark your disc or slides (your name, session and speaker order) so the slides can be returned to you.

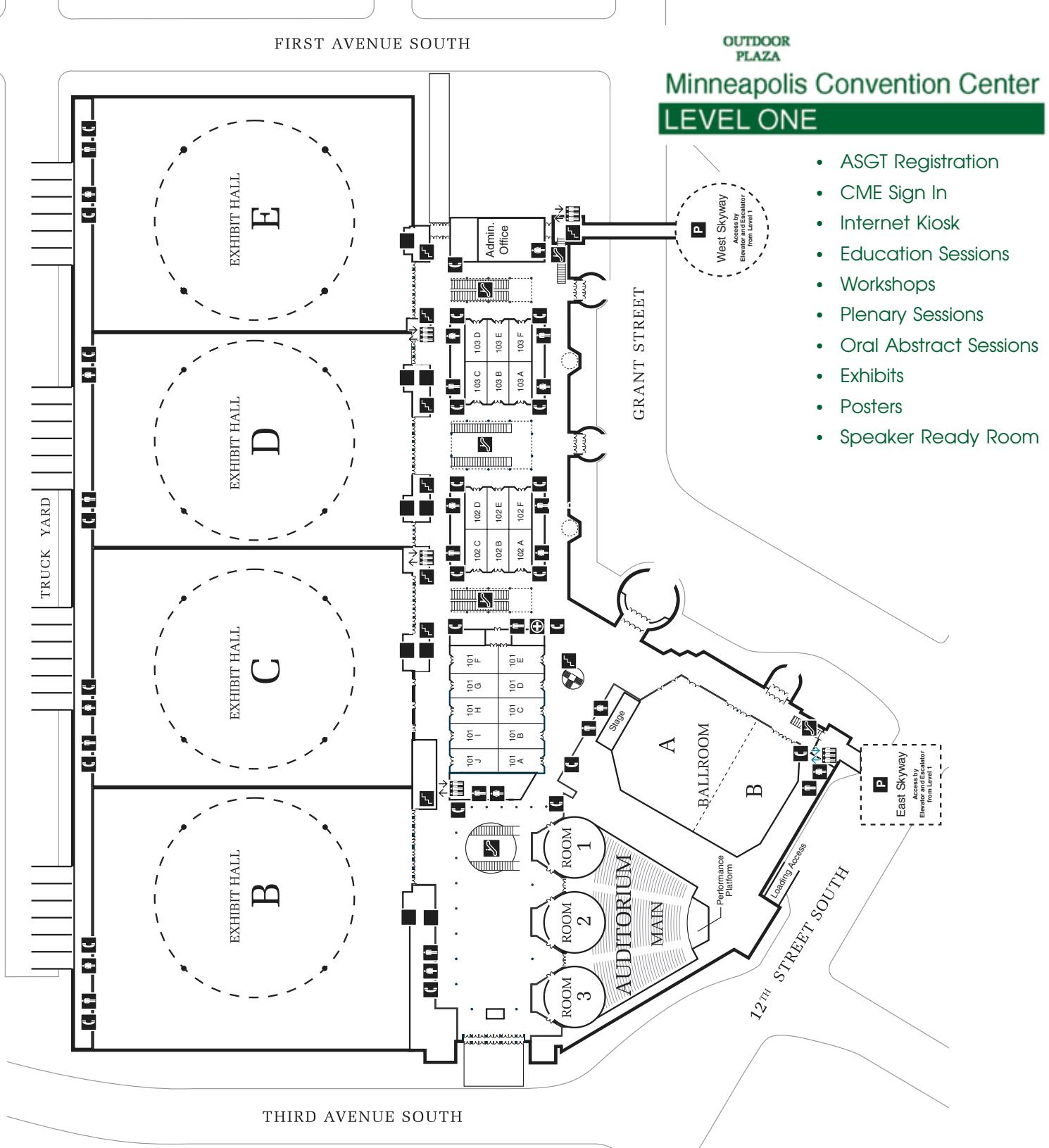
### Speaker Ready Room Hours

Wednesday, June 2	10:00 am – 9:00 pm
Thursday, June 3	6:30 am – 7:00 pm
Friday, June 4	7:00 am – 7:00 pm
Saturday, June 5	7:00 am – 6:00 pm
Sunday, June 6	7:30 am – 12:00 pm

# The American Society of Gene Therapy's 7th Annual Meeting final program

2004

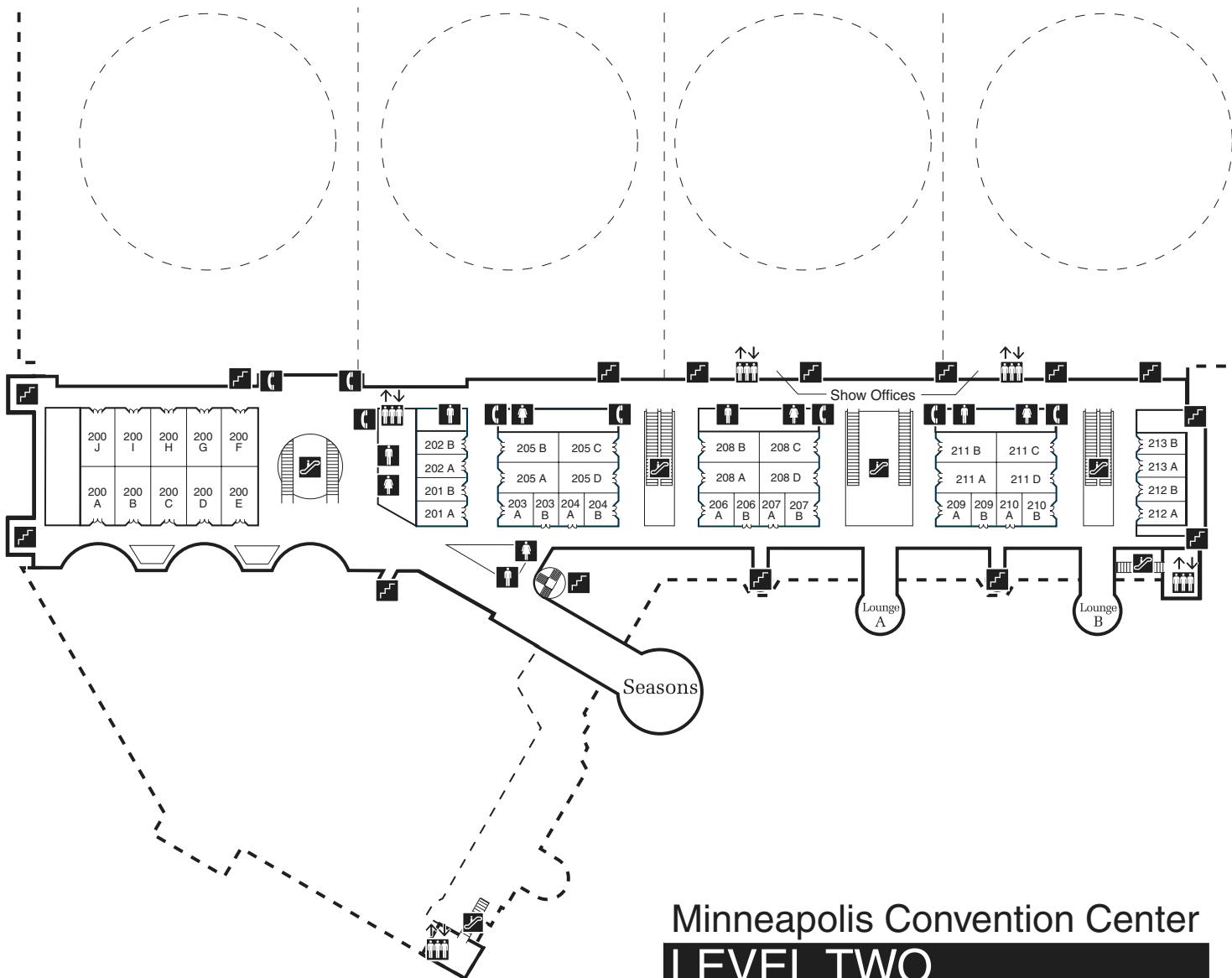
## Minneapolis Convention Center Floor Plan: Level One



# The American Society of Gene Therapy's 7th Annual Meeting final program

2004

## Minneapolis Convention Center Floor Plan: Level Two



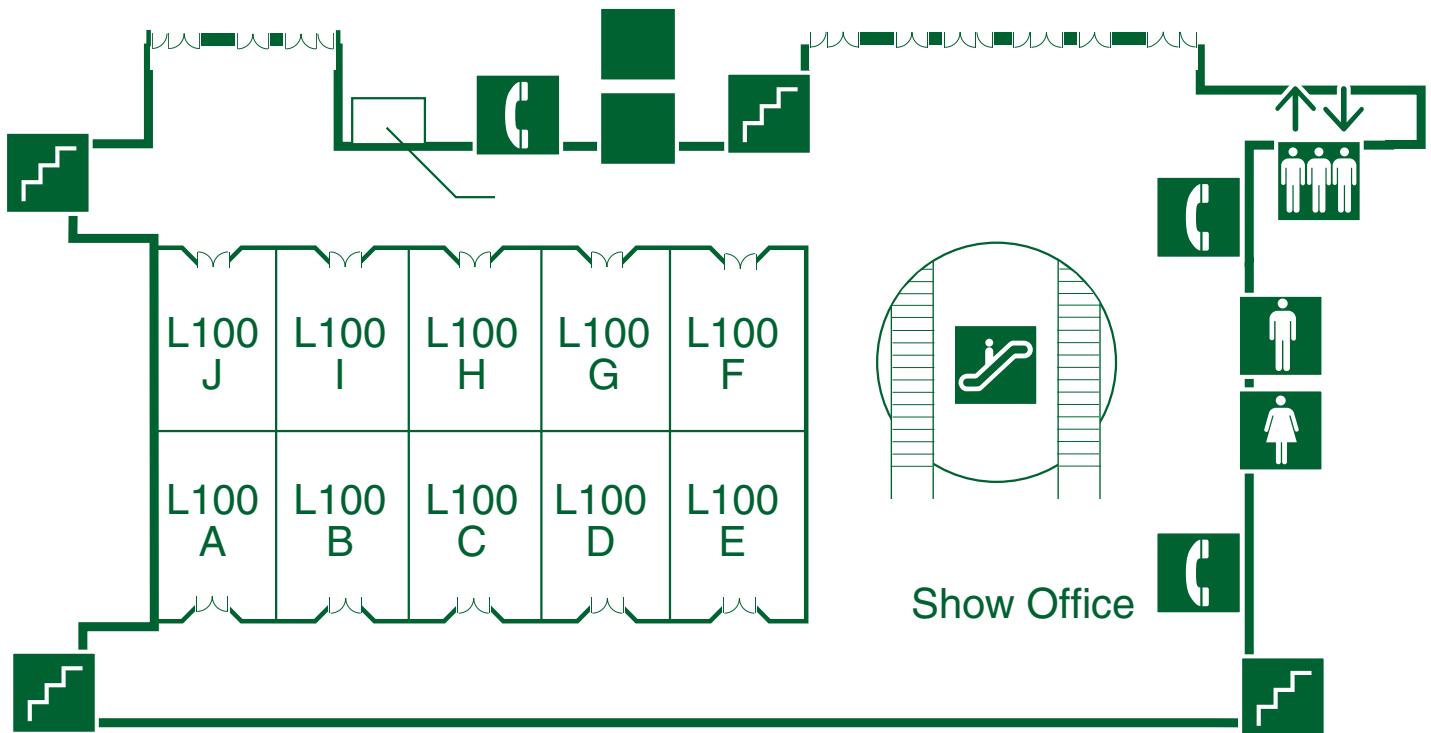
## Minneapolis Convention Center LEVEL TWO

- Internet Kiosk
- Education Sessions
- Corporate Symposia
- Workshops
- Oral Abstract Sessions
- Press Room

# The American Society of Gene Therapy's 7th Annual Meeting final program

2004

## Minneapolis Convention Center Floor Plan: Lower Level



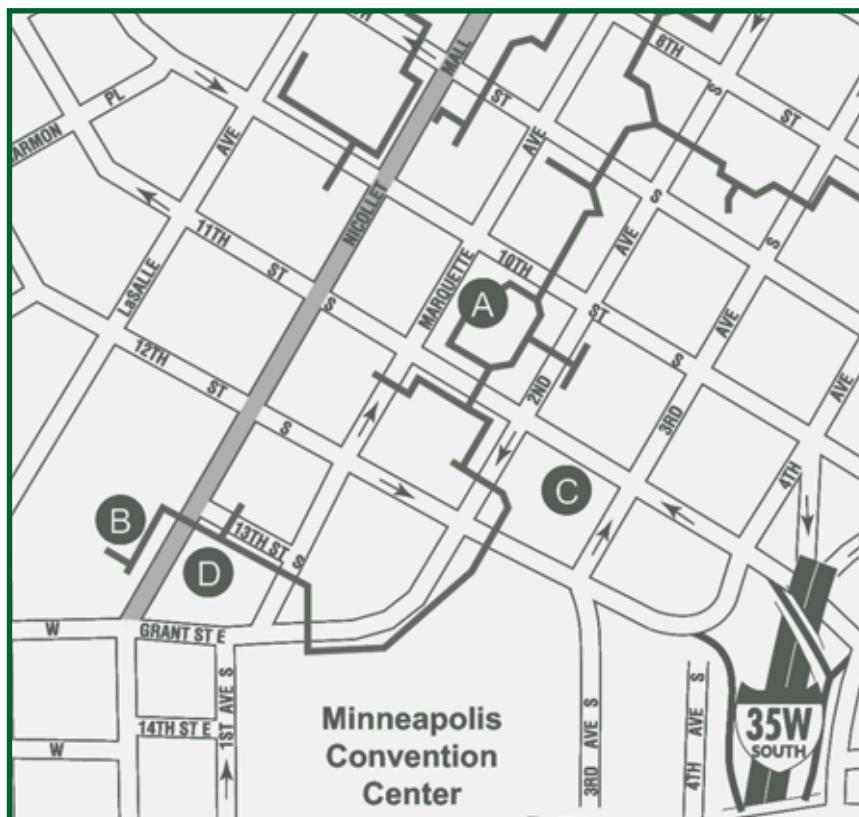
Minneapolis Convention Center  
**LOWER LEVEL**

- Meet-the-Investigator Sessions

# The American Society of Gene Therapy's 7th Annual Meeting final program

# 2004

## Hotel Information



### A. Hilton Minneapolis

1001 Marquette Avenue South  
Minneapolis, MN 55403-2440  
Phone: 612-376-1000

### B. Hyatt Regency Minneapolis (Headquarters Hotel)

1300 Nicollet Mall  
Minneapolis, MN 55403  
Phone: 612-370-1234

### C. Holiday Inn Express Hotel & Suites

225 South Eleventh Street  
Minneapolis, MN 55403-2520  
Phone: 612-341-3300

### D. Millennium Hotel

1313 Nicollet Mall  
Minneapolis, MN 55403-2697  
Phone: 612-332-6000

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### 2004 Program-at-a-Glance

#### Wednesday, June 2

Noon – 8:00 pm	Attendee Registration Open
2:00 pm – 3:45 pm	Pre-Meeting Session: Programs of Excellence in Gene Therapy
2:00 pm – 3:45 pm	Pre-Meeting Session: Understanding and Planning Biodistribution and Toxicology Studies
4:00 pm – 5:30 pm	Education Sessions
5:30 pm – 6:45 pm	Dinner Break
6:45 pm – 8:15 pm	Education Sessions
8:30 pm – 10:00 pm	Education Sessions

#### Thursday, June 3

7:00 am – 5:00 pm	Attendee Registration Open
8:00 am – 9:00 am	George Stamatoyannopoulos Lecture (Harriet L. Robinson, PhD) and Excellence in Research Awards
9:15 am – 12:30 pm	Workshops
10:00 am – Noon	Poster Setup by Authors
Noon – 7:30 pm	Poster Viewing
12:30 pm – 1:30 pm	Meet-the-Investigator Sessions
1:45 pm – 4:15 pm	Oral Abstract Sessions
3:00 pm – 7:30 pm	Exhibit Hall Open
4:00 pm – 7:30 pm	Poster Session, Exhibits, and Welcome Reception
7:00 pm – 10:00 pm	NIH Funded Resources in Support of Gene Therapy Symposium
7:00 pm – 10:00 pm	Sangamo BioSciences Corporate Symposium – "Zinc Finger DNA-Binding Proteins for Gene Regulation and Repair: A Novel Class of Human Therapeutics" Chair: Sir Aaron Klug

#### Friday, June 4

7:00 am – Noon	Poster Setup by Authors
7:30 am – 5:00 pm	Attendee Registration Open
8:00 am – 10:00 am	Scientific Symposia
10:15 am – 12:15 pm	Scientific Symposia
Noon – 7:00 pm	Poster Viewing
12:30 pm – 1:30 pm	Meet-the-Investigator Sessions
1:45 pm – 4:15 pm	Oral Abstracts Sessions
3:00 pm – 7:00 pm	Exhibit Hall Open
4:00 pm – 7:00 pm	Poster Session, Exhibits, and Networking Reception
4:30 pm – 5:15 pm	ASGT Business Meeting
7:00 pm – 10:00 pm	VIRxSYS Corporate Symposium – "Lentiviral Vectors" Chair: Boro Dropulic, PhD

#### Saturday, June 5

7:00 am – Noon	Poster Setup by Authors
7:30 am – 5:00 pm	Attendee Registration Open
8:00 am – 10:00 am	Scientific Symposia
10:15 am – 12:15 pm	Oral Abstract Sessions
Noon – 7:00 pm	Poster Viewing
12:15 - 1:15 pm	Meet-the-Investigator Sessions
1:30 pm – 4:00 pm	Presidential Symposium (Gordon Keller, PhD, and Catherine Verfaillie, MD) Presidential Address and Presentation of Top Abstracts
3:00 pm – 7:00 pm	Exhibit Hall Open
4:00 pm – 7:00 pm	Poster Session, Exhibits, and Networking Reception

#### Sunday, June 6

7:30 am – Noon	Attendee Registration Open
9:00 am – Noon	Oral Abstract Sessions
Noon	7th Annual Meeting Concludes

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Wednesday, June 2

#### Pre-Meeting Sessions

##### Programs of Excellence in Gene Therapy

2:00 pm – 3:45 pm

Room: 205 A-D

###### Co-Chairs

Sonia Skarlatos, PhD

George Stamatoyannopoulos, MD

###### Speakers

Joseph C. Glorioso, PhD - *The Pittsburgh PEGT Cardiovascular Disease Center*

Neil R. Hackett, PhD - *Capsid Modified Adenoviral Vectors for Anti-Pseudomonas Vaccination*

Hans-Peter Kiem, MD - *Stem Cell Gene Transfer and Selection: Implications for the Treatment of Genetic Diseases*

Katherine A. High, MD - *Novel Therapeutic Approach for Hemophilia Using Gene Delivery of an Engineered Secreted Activated FVII*

##### Understanding and Planning Biodistribution and Toxicology Studies

2:00 pm – 3:45 pm

Room: 211 A-D

###### Chair

Larry A. Couture, PhD

###### Speakers

Joy Cavagnaro, PhD, DABT, RAC - *Considerations in Study Design: Optimizing Value Added for Communicating Risk*

John G. Page, PhD, DABT - *Planning and Design of Preclinical Toxicology and Biodistribution Studies*

Theresa Chen, PhD - *Preclinical Assessment of Gene Therapy Products: CBER Perspective*

Lorraine Matheson - *Accessing and Contributing to Available Database Resources for Biodistribution/Toxicology Studies*

#### Education Session I

4:00 pm – 5:30 pm

##### ES100 - Retrovirus Vectors

Room: 205 A-D

Retroviral vectors created on the basis of simple gammaretroviruses such as murine leukemia viruses (MLV) represent a well characterized but still surprising tool for stable gene transfer. Efficiency and safety of these vectors depend on the multiplicity of insertions per host genome, on the pool size and specific targeting of the relevant cell type, and on the choice of appropriate cis-acting elements of the retroviral transgene. Detailed studies of retroviral vector elements are ongoing to improve the performance of current vectors, and to derive mechanisms that are of principal interest for the design of future recombinant vectors. Further progress in retroviral vector design will greatly rely on the correct choice and proper integration of non-MLV sequences in transgenes and particles, and may finally lead to the construction of ideal ferries for stable transgene insertion.

###### Chair

Christopher Baum, MD

###### Speakers

Christopher Baum, MD - *Simple Retroviruses: Not So Simple Vectors*

Didier Trono, MD - *Lentiviral Vectors for Basic Studies and Therapeutic Purposes*

Luigi Naldini, MD, PhD - *Lentiviral Vectors*

##### ES101 - AAV Vectors

Room: 102 A-C

Adeno-associated virus (AAV) is emerging as a popular vector system for long term gene transfer. This workshop will present the basic biology of AAV and review its utility in gene therapy applications. The session will begin with talks that define the biology of the virus and the differences between some of the serotypes that are commonly used as vectors for gene transfer. Other talks will discuss the transduction pathway of the vectors in the cell, trafficking events that can limit vector transduction, and vector production and preparation.

###### Chair

Jay A. Chiorini, PhD

###### Speakers

Jay A. Chiorini, PhD - *The ABCs of AAV*

Guangping Gao, PhD - *Primate Derived AAVs as Efficient Gene Transfer Vectors: Virus Evolution and Vector Development*

Giovanni Di Pasquale, PhD - *What AAV Needs to Express the Transgene: AAV/Host Cell Interactions*

Richard O. Snyder, PhD - *Recombinant AAV Vector Manufacturing*

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Wednesday, June 2

#### ES102 - Adenovirus Vectors

Room: 102 D-F

The goal of the Adenovirus Education Session is to survey the current state-of-the-art in our development and use of adenovirus gene transfer vectors. The session will begin with a discussion of the molecular biology of adenovirus including the production of replication -deficient vectors and packaging cell lines, the benefits and deficits of deletions of subsets or all the viral genes in the vector, the logic behind conditionally replicating adenoviruses, and variations relating to the Transgene expression cassette. The second topic of discussion will address the efficiency with which adenovirus interacts with target cells to deliver its genome to the nucleus. This discussion will include the basic infection pathway, altered vector tropism, variables relating to target cell type and physiology, and *in vivo* vector trafficking. The final topic of the session will be the interaction between adenovirus and the immune system including innate immunity, acquired immunity, and strategies for circumventing immune system barriers.

*Chair*

Philip L. Leopold, PhD

*Speakers*

Philip L. Leopold, PhD - *Adenovirus Education Session*

James M. Wilson, MD, PhD - *Innate and Required Immunity to Adenoviral Vectors*

Neil R. Hackett, PhD - *Molecular Biology of Adenovirus Vectors*

#### ES103 - Non-Viral Vectors

Room: 103 A-C

In seeking to develop non-viral vector systems for nucleic delivery, researchers aim to replace viral vectors with physical techniques or chemistry-based self-assembly complexes as the preferred means for nucleic delivery. So far this aim has not yet been realized due to a number of significant problems that continue to frustrate research into the development of efficient non-viral vectors for nucleic acid delivery. However, these problems are approaching solution with a number of non-viral systems currently under development. Therefore, the overarching objective of this education session is to give a realistic impression of non-viral vector systems not only in terms of "where we are now" but "where we will be soon". In this way the participant will be able to appreciate that non-viral vectors are not as far away from viral vectors as is commonly believed, and that with their inherent advantages there is every likelihood going forward that non-viral vectors can compete favorably with viral vectors for applications in clinical gene therapy.

*Chair*

Andrew D. Miller, PhD

*Speakers*

Andrew D. Miller, PhD - *Gene Therapy Needs Robust Synthetic Non-Viral Platform Technologies*

Yuhong Xu, PhD - *Gene Delivery by Physical Methods*

Ian MacLachlan, PhD - *Special Considerations for the Development of Nucleic Acid Based Drugs*

#### ES104 - Immunology

Room: 200 A-C

*In vivo* delivery of genes for therapeutic purposes and vaccines induces immune responses directed both against the vector as well as the gene-product. The goal of this educational session is to provide an overview of the role of immune responses in strategies for development of vector systems for gene therapy and vaccines. The overview of the immune response will include innate immunity, antigen processing and presentation, lymphocyte activation, induction of effector responses, and regulation of the immune responses. There will be a presentation and discussion on the use of gene transfer to induce tolerance to therapeutic or transplant antigens. Several exciting new approaches involving mechanisms of deletion, energy, or suppression will be discussed. In addition experiences of immunologic factors influencing efficacy, and safety in clinical trials with viral vectors will be reviewed.

*Chair*

Narendra Chirmule, PhD

*Speakers*

Narendra Chirmule, PhD - *Immune Responses to Vectors and Transgenes for Gene Therapy and Vaccines*

Roland W. Herzog, PhD - *Using Gene Transfer to Induce Immune Tolerance*

Ben-Gary Harvey, MD - *Immunologic Factors in Clinical Trials with Viral Vectors*

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Wednesday, June 2

#### ES105 - Expression Cassette Design

Room: 200 D-G

Essentially all gene therapy applications require functional expression of a transferred gene. This education session will focus on the design of gene expression cassettes for this purpose. We will start with an overview of the basic components required to generate a functional gene expression cassette, as well as methods for reducing the effects of flanking chromatin on expression of integrating gene transfer vectors. This will be followed by a review of the various methods for optimizing expression from gene transfer vectors. The session will conclude with a review of the various methods for regulating the level of gene expression from engineered expression cassettes.

*Chair*

David W. Emery, PhD

*Speakers*

David W. Emery, PhD - *Basic Expression Cassette Elements*

Punam Malik, MD - *Vector Design*

Victor M. Rivera, PhD - *Regulated Gene Expression*

#### ES106 - RNA Interference

Room: 211 A-D

RNA interference refers to a cells natural ability to silence gene expression using short interfering RNAs (siRNAs). Inhibitory RNAs are derived from larger precursors transcribed from the genome, or introduced into cells artificially. The power of RNAi has recently been harnessed by the bench scientist to selectively inhibit the expression of diverse gene products to ascertain biological function, or as therapeutic tools for silencing pathogenic protein expression. This session will provide an overview of the protein complexes involved in RNAi, and will also illustrate various methods to accomplish RNAi in cells, tissues, and animals.

*Chair*

Beverly L. Davidson, PhD

*Speakers*

Beverly L. Davidson, PhD - *RNA Interference*

Michael McManus, PhD - *Mammalian RNA Interference Pathways*

Judy Lieberman, MD, PhD - *Harnessing RNA Interference for Therapy*

#### ES107 - Intracellular Trafficking

Room: 200 H-J

The goal of this educational session is to provide an overview of the current understanding of viral trafficking during infection. "State-of-the-art" cell biological techniques have recently provided new insights into the mechanisms that allow lentiviruses, adenoviruses, and adeno-associated viruses to infect non-dividing cells making them excellent vectors for gene delivery. The consequences of the pathways of cytoplasmic trafficking of different vectors systems on efficient gene delivery into different target cells and tissues will be discussed. Other specific topics will include pathways of viral entry and the importance of the microtubule network in gaining access to the nucleus in the absence of cell division.

*Chair*

Thomas J. Hope, PhD

*Speakers*

Thomas J. Hope, PhD - *Intracellular Trafficking of Retroviruses*

John F. Engelhardt, PhD - *Intracellular Trafficking of rAAV*

Joseph Zabner, MD - *Molecular and Cellular Mechanisms of Adenovirus Mediated Gene Transfer*

#### Dinner Break

5:30 pm – 6:45 pm

#### Education Session II

6:45 pm – 8:15 pm

#### ES110 - Retrovirus Vectors

Room: 205 A-D

See description for Session 100

*Chair*

Christopher Baum, MD

*Speakers*

Christopher Baum, MD - *Simple Retroviruses: Not So Simple Vectors*

Didier Trono, MD - *Lentiviral Vectors for Basic Studies and Therapeutic Purposes*

Luigi Naldini, MD, PhD - *Lentiviral Vectors*

# The American Society of Gene Therapy's 7th Annual Meeting final program

# 2004

## Wednesday, June 2

### ES111 - AAV Vectors

Room: 102 A-C

See description for Session 101

*Chair*

Jay A. Chiorini, PhD

*Speakers*

Jay A. Chiorini, PhD - *The ABCs of AAV*

Guangping Gao, PhD - *Primate Derived AAVs as Efficient Gene Transfer Vectors: Virus Evolution and Vector Development*

Giovanni Di Pasquale, PhD - *What AAV Needs to Express the Transgene: AAV/Host Cell Interactions*

Richard O. Snyder, PhD - *Recombinant AAV Vector Manufacturing*

### ES112 - Adenovirus Vectors

Room: 102 D-F

See description for Session 102

*Chair*

Philip L. Leopold, PhD

*Speakers*

Philip L. Leopold, PhD - *Adenovirus Education Session*

James M. Wilson, MD, PhD - *Innate and Required Immunity to Adenoviral Vectors*

Neil R. Hackett, PhD - *Molecular Biology of Adenovirus Vectors*

### ES113 - Vector Integration

Room: 200 D-G

Integrating vector systems use the capacity of their parent viruses to insert genetic information stably into the genome of target cells, which makes them an attractive tool for the genetic modification of stem cells and other cell types with intended long term biological activities. Integrating viruses as well as their vector derivatives lead to mostly mono-allelic modification of their target DNA, which can be a useful molecular marker for the identification of target cell clone activity *in vivo*, but may also have side effects by gene activation or gene disruption. The topics of this session will include a review of several of the most important integrating vector systems, of the characteristics of their particular insertion process, and of the molecular assays for the detection and characterization of vector integration. There will be a discussion of the changes vector integration can induce in chromosomal target DNA and of state-of-the-art strategies to avoid unwanted interferences of integrating virus vector elements with their genetic environment.

*Chair*

Christof von Kalle, MD

*Speakers*

Christof von Kalle, MD - *Vector Integration in Hematopoietic Stem Cells*

Frederic D. Bushman, PhD - *Retroviral Integration*

Daniel G. Miller, MD, PhD - *AAV Vector Integration: Associated Chromosomal Changes and Mechanism*

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Wednesday, June 2

#### ES114 - Gene Transfer in the Liver

Room: 211 A-D

The liver is an excellent target for gene therapy because of the large number of genetic diseases that involve liver proteins, the ready access of the liver from the blood, the presence of receptors for most viral vectors on the hepatocyte, and the fact that liver cells have a long lifespan. This educational session will review the myriad of diseases that can be approached with liver gene therapy. The ability of AAV, retroviral, adenoviral, and plasmid vectors to transfer genes into the liver, as well as emerging issues related to stem cell based therapies will be discussed. Finally, the mechanism of liver growth and development will be reviewed, as long-term efficacy of gene therapy requires that either a long-lived cell or a stem cell be transduced. This session will highlight the outstanding potential for liver-directed gene therapy to permanently correct congenital and acquired genetic disorders.

*Chair*

Mark A. Kay, MD, PhD

*Speakers*

Mark A. Kay, MD, PhD - *Vectors and Strategies for Gene Transfer into Liver*

Katherine Ponder, MD - *Retroviral Vectors for Liver Gene Therapy*

Cary Harding, MD - *Physiologic Factors that Affect the Therapeutic Threshold in Liver Gene Therapy*

#### ES115 - Gene Transfer for the Respiratory Tract

Room: 103 A-C

This session will address recent developments in gene therapy for respiratory diseases. The emphasis will be on airway disease applications. The speakers will discuss options in vectors for pulmonary applications and strategies to approach barriers to gene transfer in the respiratory epithelium. The results from recent clinical trials and how they are shaping design of future trials will also be discussed.

*Chair*

Paul B. McCray, Jr, MD

*Speakers*

Paul B. McCray, Jr, MD - *Barriers to Airway Gene Transfer and Strategies to Overcome Them*

Wanda O'Neal, PhD - *Development of Gene Transfer Vectors for Pulmonary Disease*

Stephen Hyde, PhD - *Clinical Trials of Gene Therapy for Respiratory Disease*

#### ES116 - Cardiovascular Gene Transfer

Room: 200 A-C

Diseases of the cardiovascular system comprise some of the commonest and most serious health problems in our society. Gene therapy treatments are being developed for many diseases of the heart and blood vessels. This session will review the relevant basic biology of the cardiovascular system, including the types of cells that could be modified by gene therapy and their roles in pathological conditions. Cardiovascular diseases that are currently targets for gene therapy approaches will be discussed. Techniques for gene delivery and approaches to the selection of therapeutic genes will also be covered.

*Chair*

David A. Dichek, MD

*Speakers*

David A. Dichek, MD - *Gene Transfer to Blood Vessels*

Roger J. Hajjar, MD - *Targeted Cardiac Gene Transfer*

Walter J. Koch, PhD - *Intra Coronary Myocardial Gene Delivery for Heart Failure*

#### ES117 - Gene Transfer in the Eye

Room: 200 H-J

The eye is an ideal organ for gene therapy studies for many reasons, including its size, favorable immunological characteristics, availability of a variety of gene delivery approaches, access to animal models, and ability to measure effects of treatment in a non-invasive fashion. This session will concentrate on the retina as a target for gene therapy. It will discuss practical problems in testing gene therapy in animal models of retinal disease. It will also discuss real and theoretical applications of somatic gene manipulation for studying the biology of the healthy retina.

*Chair*

Jean Bennett, MD, PhD

*Speakers*

Jean Bennett, MD, PhD - *From Mice to Monkeys: Ocular Gene Transfer for Generation of Animal Models and for Therapy*

Tomas S. Aleman, MD - *How Do You Quantify Vision in Genetic Retinal Degenerations?*

Fong-Qi Liang, MD, PhD - *Intraocular Delivery of Viral Vectors*

*Break*

8:15 pm – 8:30 pm

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

**Wednesday, June 2**

### **Education Session III**

8:30 pm – 10:00 pm

#### **ES120 - Infectious Diseases**

Room: 102 A-C

This education session will provide a background for understanding the many roles RNA interference (RNAi) can have in gene regulation. There will be discussions of the RNAi mechanism and several different potential modes of action. The major focus of the session will be applications of RNAi for the treatment of infectious diseases. Strategies for targeting and delivery of short hairpin genes and synthetic small interfering RNAs (siRNAs) will be presented, along with preclinical data using RNAi for treating and preventing viral infections. The session attendees will be able to evaluate the potential for using RNAi in treating viral infections, the potential limitations and an understanding of the mechanisms of this important biological regulatory process.

*Chair*

John J. Rossi, PhD

*Speakers*

John J. Rossi, PhD - *The Nuts and Bolts of RNAi for Infectious Diseases*

Mark A. Kay, MD, PhD - *RNAi Strategies for Viral Infection*

Daniel Boden - *The Potential of RNA Interference to Treat HIV-1 Infection*

#### **ES121 - Imaging in Gene Therapy**

Room: 102 D-F

As gene therapy moves into the clinic, it will be essential to monitor the functioning of gene therapy vectors in patients. This session will provide an overview of novel imaging techniques that can be used to image disease progression, the functioning of gene therapy vectors, and the efficiency of gene therapy in live experimental individuals. Importantly, this session will present advances in various levels of non-invasive monitoring of gene expression: from fluorescent proteins, PET-imaging, and luciferase optical imaging. Advantages and limitations of each method will be discussed, and discussions will encompass how best to address various experimental challenges. Participants are encouraged to challenge the speakers to provide imaging advice and experimental approaches to participants' own scientific questions.

*Chair*

Pedro Lowenstein, MD, PhD

*Speakers*

Pedro Lowenstein, MD, PhD - *Imaging in the CNS*

Kurt R. Zinn, DVM, PhD - *SPECT/CT and Multimodality Imaging in Gene Therapy Research*

Andreas H. Jacobs, MD - *Imaging-Guided Gene Therapy in the CNS*

#### **ES122 - Oncolytic Viruses**

Room: 205 A-D

The speakers in the session will be experts on three oncolytic vector systems: human adenovirus type 5, Herpes Simplex Virus (HSV), and an RNA virus, either Vesicular Stomatitis Virus (VSV) or Measles Virus (MV). Each speaker will provide general background on adenovirus, HSV, and either VSV or MV oncolytic vectors. Following the background introduction, the rationale for developing adenovirus, HSV, and VSV or MV oncolytic vectors will be discussed. Each speaker will review previous research on each of these vectors, and then they will provide highlights of their own research with the goal of educating the audience about the different vector systems.

*Chair*

William Wold, PhD

*Speakers*

William Wold, PhD - *Adenovirus Vectors for Cancer Gene Therapy*

Robert L. Martuza, MD - *Oncolytic Herpes Simplex Vectors for Cancer Therapy*

Stephen J. Russell, MD, PhD - *Measles*

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Wednesday, June 2

#### ES123 - NIH Grantsmanship & Funding

Room: 103 A-C

The goal of this session is to acquaint participants with the grant review and funding process at the NIH. Participants will get advice on what contributes to a successful grant application from NIH staff. Staff will also provide information on funding opportunities in gene therapy as well as those available to support training. There will be an opportunity for questions during the panel discussion. This session will be aimed at new investigators but should be informative for any investigator planning to submit an NIH grant application.

*Chair*

Terry R. Bishop, PhD

*Speakers*

Terry R. Bishop, PhD

Sonia Skarlatos, PhD - *Role of NIH Program Staff*

Brendan Lee, MD, PhD - *NIH Grant Review: The Study Section Member's Perspective*

#### ES124 - Regulatory Issues

Room: 211 A-D

The goal of this session is to identify key elements of the preclinical testing, clinical-grade manufacturing, and clinical evaluation of gene therapy products as they proceed through the Investigational New Drug (IND) process. The session will highlight points that frequently lead to confusion for IND sponsors and investigators. In particular, the session will focus on which aspects of the supporting preclinical data and production processes are critical to have in place prior to IND submission, and which may be further developed between the time of the initial application for investigational use and the ultimate application for clinical licensure. There will also be an overview of the current expectations for long-term clinical follow-up of human subjects enrolled in gene therapy trials. Finally, an overview of the responsibilities of the Principal Investigator will be reviewed both in the context of corporate-sponsored IND's and in the context of investigator or institution-sponsored IND's.

*Chair*

Terence R. Flotte, MD

*Speakers*

Maritza C. McIntyre, PhD - *What Aspects of GMP Do I Need?*

Theresa Chen, PhD - *What Preclinical Data Do I Need for an IND Submission?*

Daniel Rosenblum, MD - *What Clinical Data Does the FDA Require for Long-Term Follow-Up?*

Terence R. Flotte, MD - *What is the Principal Investigator Responsible for?*

### Thursday, June 3

#### 200 - George Stamatoyannopoulos Lecture

8:00 am – 9:00 am

Room: Auditorium

*Chair*

Katherine A. High, MD

*Speaker*

Harriet L. Robinson, PhD - *MultiProtein DNA/MVA Vaccine for HIV/AIDS*



Excellence in Research Awards for Students and Post Doctoral Fellows Announced

#### Coffee Break

9:00 am – 9:15 am

#### Workshops

9:15 am – 10:45 am

#### WS210 - American Society of Transplantation: The Nexus of Cell Transplantation and Gene Therapy

Room: 200 A-C

*Co-Chairs*

Daniel R. Salomon, MD

Donald B. Kohn, MD

*Speakers*

Jonathan S. Bromberg, MD, PhD - *Organ and Cell Transplantation: Challenges and Opportunities*

Edward Baetge, PhD - *Applications of Stem Cells for the Generation of Pancreatic Islet Cells*

#### WS211 - Cancer: Surrogate Markers and Interval End Points

Room: 102 A-F

*Chair*

Akseli E. Hemminki, MD, PhD

*Speakers*

Stephen J. Russell, MD, PhD - *Noninvasive Monitoring of Oncolytic Viruses*

Akseli E. Hemminki, MD, PhD - *Non-Invasive Monitoring of Oncolytic Adenovirus Replication*

Long P. Le, PhD - *Genetic Labeling System for Monitoring Oncolytic Adenoviruses*

Donald Buchsbaum, PhD - *Imaging of Gene Expression*

Savio L.C. Woo, PhD - *Should Gene Therapy be Different from Chemotherapy and Antibody Therapy?*

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Thursday, June 3

#### WS212 - Cardiovascular: Targeted Gene Delivery for Myocardial and Vascular Disease

Room: 103 A-C

Co-Chairs

Barry J. Byrne, MD, PhD

Frank J. Giordano, MD

Speakers

Xiao Xiao, PhD - Therapeutic Gene Delivery in Heart of Muscular Dystrophin Animal by Novel AAV Vectors  
Walter J. Koch, PhD - IntraCoronary Gene Delivery for Heart Failure

Andrew H. Baker, PhD - Engineering Viral Vectors for Vascular Gene Therapy

Kenneth H. Warrington, Jr, PhD - Alterations in AAV Capsids for Tissue Tropism

Vijay K. Yechoor, MD - Gene Therapy for Familial Hypercholesterolemia and Diabetes Mellitus

#### WS213 - Genetic Disease: Large Animal Models of Genetic Disease

Room: Auditorium Room 1

Chair

Mark Haskins, VMD, PhD

Speakers

Patrick Aebsicher, MD - Modeling CNS

Neurodegeneration by Overexpression of Disease-Causing Proteins Using Viral Vectors

Timothy C. Nichols, MD - Molecular Defects in Hemophilia A, Hemophilia B, and von Willebrand Disease Dogs

Joe Kornegay, DVM, PhD - Use of the Golden Retriever Muscular Dystrophy Model in Developmental Therapeutics

Gustavo D. Aguirre, VMD, PhD - Long-Term Restoration of Visual Cell Function and Structure With AAV-Mediated Gene Therapy in Canine LCA

Mark Haskins, VMD, PhD - Gene Therapy in Animal Models of Mucopolysaccharidosis

#### WS214 - Immunology of Gene Therapy: Biology of Genetically Modified Antigen-Presenting Cells (APCs)

Room: Auditorium Room 3

Chair

Karin Jooss, PhD

Speakers

Isabelle Riviere, PhD - Activation and Expansion of Specific Cytotoxic T-Lymphocytes Using Artificial Antigen Presenting Cells

Carl H. June, MD - Genetically Engineered Artificial Antigen Presenting Cells

Paul D. Robbins, PhD - Genetically Modified APC for Treatment of Autoimmune Disease

Katie Whartenby, PhD - Gene-modified Hematopoietic

#### Stem Cells to Enhance Immunity

#### WS215 - Industrial Liaison: Commercializing Gene Therapy - What Will It Take?

Room: 205 A-D

Chair

Stephen M.W. Chang, PhD

Speakers

Samuel C. Wadsworth, PhD - Choosing the Right Gene Therapy Target, A Balancing Act

Philip D. Noguchi, MD - FDA is Not the Gatekeeper

Douglas J. Jolly, PhD - Expectations and Reality in Gene Therapy

Robert E. Sobol, MD - Advexin-Late Stage Development of a Tumor Suppressor Gene Therapy

#### WS216 - Non-Viral: Development and Application of Non-Viral Vectors

Room: 211 A-D

Co-Chairs

Seng H. Cheng, PhD

Lluis M. Mir, DSc

Speakers

Lluis M. Mir, DSc - Optimized Safe DNA Electroporation in Muscles and Tumors Using Combination of Pulses

David B. Rozema, PhD - Endosomolysis by Reversible Inhibition of a Membrane-Active Agent

Mark J. Cooper, MD - Highly Effective DNA Nanoparticles for Intrapulmonary Gene Delivery

Carol H. Miao, PhD - Current Status of Nonviral Gene Therapy for Hemophilia

Alain Rolland, PharmD, PhD - Development of Cationic Lipid Systems for Intramuscular Delivery of Plasmid-Based Vaccines: Application to an Anthrax Vaccine

#### WS217 - Respiratory Tract: Stem Cell Plasticity-Use of Bone Marrow-Derived Cells for Lung Diseases

Room: 208 A-D

Co-Chairs

Daniel Weiss, MD, PhD

Guoshun Wang, DVM, PhD

Speakers

Daniel Weiss, MD, PhD - Re-population of CFTR KO Mouse Lungs with Adult Marrow-Derived Cells

Susan D. Reynolds, PhD - Tissue-Specific Stem Cells but not Bone Marrow-Derived Cells Reconstitute the Conducting Airway Epithelium Following Secretory Cell Depletion

Xiaoming Liu, PhD - CFTR mRNA Gene Correction in Airway Stem Cells Reduces CFTR-mediated Developmental Toxicity

Luis Ortiz, MD - From Bone Marrow Mesenchyma to Alveolar Epithelium

Guoshun Wang, DVM, PhD - Potential of Bone Marrow Stromal Stem Cells for Cystic Fibrosis Therapy

Jeffrey L. Spees, PhD - Adult Stem Cells and Bone Marrow-Derived Cells in Lung Injury: Differentiation and Cell Fusion

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

**Thursday, June 3**

### **Break**

10:45 am – 11:00 am

### **Workshops**

11:00 am – 12:30 pm

#### **WS220 - Gene-Based Vaccines: Influence of Vector-Induced Innate Immunity of Gene-Based Vaccines**

Room: Auditorium Room 1

##### *Chair*

Eyal Raz, MD

##### *Speakers*

Eyal Raz, MD - *Immunostimulatory DNA: A Double-Edged Sword for Genetic Vaccination*

Art Krieg, MD - *Activation of TLR9 by CpG Motifs: Preclinical and Clinical Studies*

Daniel A. Muruve, MD - *The Innate Immune Response to Viral Vectors: The Benefits of Acute Inflammation*

Paula Pitha-Rowe, PhD - *Enhancement of Antigen Recognition by Factors Regulating the Innate Immune Responses*

#### **WS221 - Hemopoietic: Harnessing Novel Sources of Stem Cells for Therapy**

Room: 102 A-F

##### *Chair*

Jan Nolta, PhD

##### *Speakers*

Margaret A. Goodell, PhD - *Hematopoietic Stem Cells for Therapy of Non-Hematopoietic Disease?*

Fabio Rossi, MD, PhD - *The Origin of Circulating Myogenic Progenitors*

Thalia Papayannopoulou, MD - *Bone Marrow Homing of In Vitro Incubated Stem Cells: What Has Changed and Does It Matter?*

Peter Quesenberry, MD - *The Stem Cell Continuum: Therapeutic Potentials*

#### **WS222 - International Harmonization of Regulatory Standards**

Room: Auditorium Room 3

##### *Co-Chairs*

Odile Cohen-Haguenauer, MD, PhD

Estuardo Aguilar-Cordova, PhD

##### *Speakers*

Lucio Luzzatto, MD, PhD - *Lessons From the OTC Case and From the X-SCID Case*

Odile Cohen-Haguenauer, MD, PhD - *Dissemination of Information Regarding a Major Event: Leukemia in X-SCIDS as Case-study*

Beth Hutchins, PhD - *Update on Use and Stability of an Adenovirus Reference Material*

Philip D. Noguchi, MD - *Overview of FDA Practices for Adverse Event Information*

Theodore Friedmann, MD - *Possible Approach to Enhance Harmonization*

#### **WS223 - Infectious Disease: Viral Cellular Co-Factors as Targets for Gene Therapy**

Room: 103 A-C

##### *Chair*

Didier Trono, MD

##### *Speakers*

Didier Trono, MD - *Defensive Arts: Innate Intracellular Responses Against Retroelements*

David Strayer, MD, PhD - *Exploiting Hepatitis B and C Virus Activation of Cellular Transcription Factors to Provide Conditional Expression of Antiviral Transgenes*

Mauro Giacca, MD, PhD - *Control of HIV Gene Expression by Cromatin Conformation and Transcriptional Coactivators*

Reuben S. Harris, PhD - *APOBEC3G and Immunity to Retroviruses*

#### **WS224 - Musculo-Skeletal: Designing and Planning Clinical Trials for Musculo-Skeletal Diseases**

Room: 205 A-D

##### *Chair*

Jeffrey Chamberlain, PhD

##### *Speakers*

Jon Wolff, MD - *Intravascular Delivery of Naked DNA for Muscular Dystrophy*

Jeffrey Chamberlain, PhD - *Considerations for Gene Therapy Trials in Duchenne Muscular Dystrophy*

Alan J. Nixon, BVSc, MS - *Insulin-like Growth Factor Gene Therapy - Preclinical Research Techniques*

Haim Burstein, PhD - *AAV-TNFR:Fc Gene Therapy for Inflammatory Joint Diseases*

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Thursday, June 3

#### WS225 - Neural Disorders: The Neuroimmunology of Gene Therapy

Room: 208 A-D

*Chair*

Pedro Lowenstein, MD, PhD

*Speakers*

Trevor Owens, PhD - *Cytokine and Chemokine Gene Delivery to the Central Nervous System*

John Forsayeth, PhD - *Pre-existing Immunity and AAV Delivery to the Striatum*

Howard J. Federoff, MD, PhD - *HSV Amplicons for Vaccination*

E. Antonio Chiocca, MD, PhD - *Innate Immunity to Oncolytic Viruses*

Ronald Mandel, PhD - *Clinical Implications of Immune Responses to rAAV in the Brain*

#### WS226 - Viral: DNA Vector Targeting

Room: 211 A-D

*Chair*

Stefan Kochanek, MD

*Speakers*

Joel N. Glasgow, PhD - *Incorporation of Non-human Adenovirus Fiber Domains for CAR-independent and Infectivity Enhanced Adenovirus Vectors*

Michael A. Barry, PhD - *Cell Targeting Technologies for Gene Therapy*

Michael Hallek, MD - *Receptor Targeting of AAV Vectors*

Jeffrey S. Bartlett, PhD - *Development of Targeted AAV Vector Systems for In Vivo Gene Delivery*

### Meet-the-Investigator Sessions

12:30 pm – 1:30 pm

The Meet-the-Investigator sessions are designed to provide an opportunity for participants to interact with investigators and discuss topics related to their research. A lunch will be served during the program, which will begin at 12:30 pm and last for approximately one hour.

The Meet-the-Investigator sessions require tickets for admission. Tickets for these sessions are not included in the regular registration fees to attend the meeting. Ushers will be collecting tickets at the door of each session. Tickets are available at the registration desk. Please note that space is limited.

#### MI230 - Gene Therapy for Cancer: Academic and Industry Implementation

Room: L100 A

Estuardo Aguilar-Cordova, PhD

#### MI231 - Gene and Cell Therapy for Muscular Disorders

Room: L100 B

Olivier Danos, PhD

#### MI232 - To Be Announced

Room: L100 C

Sir Aaron Klug, OM, FRS

#### MI233 - Increasing the Potency of Cancer Immunotherapeutics and Vaccines: Immunogenicity of Transgene and Anti-Vector Responses

Room: L100 D

Margaret A. Liu, MD

#### MI234 - Ups & Downs, Ins & Outs of Gene Therapy

Room: L100 E

Inder Verma, PhD

### Simultaneous Oral Presentations

1:45 pm – 4:15 pm

#### OA240 – Vector Integration and Mobilization

Room: 205 A-D

*Moderators*

Paula M. Cannon, PhD

Kyriacos A. Mitrophanous, PhD

(Abstracts #1 - #10)

#### OA241 – AAV Vectors: Genome and Capsid Modifications

Room: Auditorium Room 1

*Moderators*

Robert M. Kotin, PhD

Matthew Weitzman, PhD

(Abstracts #11 - #20)

#### OA242 – Novel Cancer Targeted Therapeutics

Room: 208 A-D

*Moderators*

Albert B. Deisseroth, MD, PhD

Murali Ramachandra, PhD

(Abstracts #21 - #30)

#### OA243 – Inborn Errors of Metabolism: Hemophilia and Other Disorders

Room: Auditorium Room 3

*Moderators*

Mark Haskins, VMD, PhD

Roland Herzog, PhD

(Abstracts #31 - #40)

#### OA244 – Neuro Gene Therapy I: New Approaches for Dominant, Recessive, Degenerative, and Autoimmune Diseases

Room: 103 A-C

*Moderators*

Howard J. Federoff, MD, PhD

Pedro Lowenstein, MD, PhD

(Abstracts #41 - #50)

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

**Thursday, June 3**

### **OA245 – Gene Regulation: Stem Cells**

Room: 102 A-C

*Moderators*

Casey Case, PhD

Sonya Franklin, PhD

(Abstracts #51 - #55)

### **OA246 – Adenovirus Biology and Disease Applications**

(Please Note: Session is 1:45 pm – 3:00 pm)

Room: 211 A-D

*Moderators*

Joseph T. Bruder, PhD

Michael Imperiale, PhD

(Abstracts #56 - #60)

### **OA247 – Other DNA Viruses**

(Please Note: Session is 3:00 pm – 4:15 pm)

Room: 211 A-D

*Moderators*

David Bartlett, MD

William Bowers, PhD

(Abstracts #61 - #65)

### **Poster Session I**

4:00 pm – 7:30 pm

Hall D

### **RNA Virus Vectors I**

(Abstracts #66 through #87)

### **Serotypes Capsids and Specific Applications**

(Abstracts #88 through #109)

### **Adenovirus Targeting**

(Abstracts #110 through #137)

### **Naked DNA-Based Gene Transfer I**

(Abstracts #138 through #157)

### **Inborn Errors of Metabolism: Hemophilia and Other Diseases**

(Abstracts #158 through #186)

### **Angiogenesis and Vascular Gene Therapy**

(Abstracts #187 through #206)

### **Central Nervous System I**

(Abstracts #207 through #234)

### **Muscle**

(Abstracts #235 through #251)

### **Cancer Immunotherapy**

(Abstracts #252 through #274)

### **Targeted Cancer Therapies I**

(Abstracts #275 through #296)

### **Gene Regulation: Methods**

(Abstracts #297 through #326)

### **Corporate Symposium CS250**

Room: 200

7:00 pm – 10:00 pm

### **Zinc Finger DNA-Binding Proteins for Gene Regulation and Gene Correction: A Novel Class of Human Therapeutics**

*Supported by Sangamo BioSciences*

*Chair*

Sir Aaron Klug, OM, FRS

*Speakers*

Sir Aaron Klug, OM, FRS - *Why Engineered Zinc Finger DNA-Binding Proteins (ZFPs)?*

Philip D. Gregory, PhD - *Specific Differential Technical Advantages of ZFPs for Therapeutic Applications*

Frank J. Giordano, MD - *ZFP TFs for the Treatment of Cardiovascular Disease*

Matthew H. Porteus, MD, PhD - *Targeted Gene Correction*

Donald B. Kohn, MD - *Therapeutic Applications of ZFP-Mediated Targeted Gene Correction*

### **Evening Symposium SS251**

#### **NIH Funded Resources in Support of Gene Therapy**

Room: 211 A-D

7:00 pm – 10:00 pm

*Chair*

Joseph C. Glorioso, PhD

*Speakers*

Rosemarie E. Aurigemma, PhD - *Developing Gene Therapy Products Through the Developmental Therapeutics Program and NCI*

Allan Shipp - New Information Resources from the NIH Office of Biotechnology Activities

Richard A. Knazek, MD - *The National Gene Vectors Laboratories - Vector Production and Toxicology Support*

Sonia Skarlatos, PhD - *NHLBI Programs of Excellence in Gene Therapy*

Terry R. Bishop, PhD - Resources from the NIDDK

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

**Friday, June 4**

### Scientific Symposia

8:00 am – 10:00 am

#### **SS300 - Hemopoietic: Cancer Stem Cell**

Room: Auditorium

*Chair*

John E. Dick, PhD

*Speakers*

Craig T. Jordan, PhD - *Characterization and Targeting of Leukemic Stem Cells*

Peter Dirks, MD, PhD, FRCSC - *Cancer Stem Cells in Human Brain Tumors*

Dennis R. Roop, PhD - *The Role of Multipotent Stem Cells in Non-Melanoma Skin Cancer*

#### **SS301 - Immunology of Gene Therapy: The Advent of Genetically Modified T Lymphocytes**

Room: 205 A-D

*Chair*

Bruce R. Blazar, MD

*Speakers*

David Klatzmann, MD, PhD - *Targeting Developing Thymocytes for Gene Therapy: Model of ZAP-70 Deficiency*

Michel Sadelain, MD, PhD - *Targeting Tumors with Genetically Enhanced T Lymphocytes*

Michael Jensen, MD - *Genetically Re-directed T-Cells for Cancer Immunotherapy: From Concept to Clinic*

Stanley Riddell, MD - *Pharmacologic Control of Adoptively Transferred T Cells in a Non-Human Primate Model*

#### **SS302 - Infectious Diseases: Emerging Infectious Diseases as Opportunities for Gene Therapy**

Room: Auditorium Room 1

*Chair*

John J. Rossi, PhD

*Speakers*

Glenn Randall, PhD - *Dissecting Hepatitis C Virus Replication with RNA Interference*

Claudio Bordignon, MD - *Stem Cell Gene Therapy for Congenital and Acquired Immunodeficiencies*

Geoff Symonds, PhD - *Trials and Tribulations in the Gene Therapy Approach to HIV/AIDS*

#### **SS303 - Non-Viral: Advances in Non-Viral Gene Transfer**

Room: 208 A-D

*Co-Chairs*

Sean M. Sullivan, PhD

Jon Wolff, MD

*Speakers*

Francis C. Szoka, Jr, PhD - *Assembly of Bioresponsive Gene Carriers*

Michele P. Calos, PhD - *Site-specific Integration for Gene Therapy*

Kam W. Leong, PhD - *Biodegradable Polymeric Gene Carriers*

Pamela Davis, MD, PhD - *Stabilized, Compacted DNA Nanoparticles Transfect Airway Epithelium In Vivo*

#### **SS304 - Respiratory Tract: Gene and Stem Cell Therapy for Lung Cancer**

Room: 211 A-D

*Co-Chairs*

Raj Batra, MD

Steven M. Albelda, MD

*Speakers*

Steven Dubinett, MD - *Dendritic Cell-Based Genetic Immunotherapy for Lung Cancer*

John J. Nemunaitis, MD - *Clinical Update of Gene Based Vaccines in Lung Cancer*

Daniel H. Sterman, MD - *Human Gene Transfer Trials for Pleural Malignancies*

John G. Hay, MD - *Replicating Adenoviral Vectors for Lung Cancer*

### Coffee Break

10:00 am – 10:15 am

### Scientific Symposium

10:15am – 12:15 pm

#### **SS310 - Cancer: Genetic Modification of Dendritic Cells**

Room: Auditorium

*Chair*

David T. Curiel, MD, PhD

*Speakers*

David T. Curiel, MD, PhD - *Targeted Adenoviral Vectors to Accomplish Dendritic Cell Immunotherapy*

Stephen Gottschalk, MD - *Gene Modified Dendritic Cells to Enable T-cell Therapies*

Eli Gilboa, PhD - *A Central Role of RNA in Developing Therapeutic Vaccines for Cancer and Infectious Disease*

James J. Mulé, PhD - *SLC Gene-Modified Dendritic Cells as a Tumor Vaccine*

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

**Friday, June 4**

### **SS311 - Cardiovascular: Cell and Gene Therapy for Heart Failure**

Room: Auditorium Room 1

*Chair*

Todd K. Rosengart, MD

*Speakers*

Todd K. Rosengart, MD - *Combined Gene and Cell Therapy for Treating Heart Disease*

Barry J. Byrne, MD, PhD - *Approaches to Cardiac Hypertrophy Using AAV Vectors*

Frank J. Giordano, MD - *Gene Regulation by Engineered Transcription Factor to Augment Cardiac Function*

### **SS312 - Embryonic Stem Cells & Tissue Engineering**

Room: 205 A-D

*Chair*

Mervin Yoder, MD

*Speakers*

George Q. Daley, MD, PhD - *Modeling Hematopoietic Transplantation with ES Cells*

Lorenz Studer, MD - *Human Embryonic Stem Cells and Therapeutic Cloning in Neural Disease*

Jennifer L. West, PhD - *Biomimetic Strategies in Vascular Tissue Engineering*

### **SS313 - Genetic Diseases: Gene Therapy for Genetic Disease Using Non-Replacement Strategies**

Room: 208 A-D

*Chair*

Mark A. Kay, MD, PhD

*Speakers*

Dieter Gruenert, PhD - *Sequence-Specific Modification of Genomic DNA with Fragments of DNA*

Beverly L. Davidson, PhD - *RNAi for Dominant Neurogenetic Diseases*

Ronald G. Crystal, MD - *Correction of Genetic Disorders at the Pre-mRNA Level by Spliceosome-mediated Trans-splicing*

Christopher Walsh, MD, PhD - *RNA Trans-Splicing and Genetic Correction*

### **SS314 - Musculo-Skeletal: New Approaches to Therapy of Musculo-Skeletal Diseases**

Room: 211 A-D

*Chair*

Michael Themis, PhD

*Speakers*

David W. Russell, MD, PhD - *Collagen Gene Targeting in Mesenchymal Stem Cells from Individuals with Osteogenesis Imperfecta*

Michael Themis, PhD - *The Fetal Approach: A Novel Therapy for the Treatment of Musculo-Skeletal Disease*

Josephine Nalbantoglu, PhD - *Dystrophin Gene Transfer*  
Terence A. Partridge, PhD - *Production of Dystrophin Protein in the Muscles of the MDX Mouse Induced by Local and Systemic Injection of Antisense Oligonucleotides*

### **SS315 - Special: Young Investigators Symposium**

Room: 200

*Chair*

Malcolm Brenner, MD, PhD

*Speakers*

Michael A. Barry, PhD - *Cell-Targeting Technologies for Gene Therapy*

Introduced by Arthur Beaudet, MD

Laurence JN Cooper, MD, PhD - *T-Cell Therapy for Malignant B Cells*

Introduced by John Zaia, MD

Matthew Weitzman, PhD - *Lessons from the Battleground of Virus - Host Interactions*

Introduced by Inder Verma, PhD

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

**Friday, June 4**

### **Meet-the-Investigator Sessions**

12:30 pm – 1:30 pm

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### **MI320- In Vivo Therapy with Stabilized Compacted DNA**

Room: L100 A

Pamela Davis, MD, PhD

### **MI321 – Immune Responses to Gene Therapy: The Final Frontier**

Room: L100 B

Pedro Lowenstein, MD, PhD

### **MI322 – Foamy Virus Vectors**

Room: L100 C

David W. Russell, MD, PhD

### **MI323 - Do You Really Want to Know What The FDA Thinks When They Say...**

Room: L100 D

Stephanie Simek, PhD

### **MI324 – Cancer Gene Therapy**

Room: L100 E

Savio LC. Woo, PhD

### **Simultaneous Oral Presentations**

1:45 pm – 4:15 pm

### **OA330 – RNA Virus Vectors: New Technologies**

Room: 205 A-D

*Moderators*

Michael Bitzer, MD, PhD

Richard G. Vile, PhD

(Abstracts #327 - #336)

### **OA331 – AAV Vectors: Serotypes and Applications**

Room: 211 A-D

*Moderators*

Joseph Rabinowitz, PhD

Arun Srivastava, PhD

(Abstracts #337 - #346)

### **OA332 – Cancer Immunotherapy**

Room: 208 A-D

*Moderators*

Catherine M. Bollard, MD

Michael Jensen, MD

(Abstracts #347 - #356)

### **OA333 – Molecular Conjugates**

Room: Auditorium Room 1

*Moderators*

Theresa Reineke, PhD

David V. Schaffer, PhD

(Abstracts #357 - #365)

### **OA334 – Infectious Diseases and Vaccines**

Room: Auditorium Room 3

*Moderators*

Philip Johnson, MD

Carl June, MD

(Abstracts #366 - #375)

### **OA335 – Gene Therapy for Musculoskeletal Diseases**

Room: 103 A-C

*Moderators*

Steve Ghivizzani, PhD

Johnny Huard, PhD

(Abstracts #376 - #385)

### **OA336 – Cardiovascular Gene Therapy**

Room: 102 A-C

*Moderators*

J. Kevin Donahue, MD

Douglas Losordo, MD

(Abstracts #386 - #395)

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

**Friday, June 4**

### Poster Session II

4:00 pm – 7:00 pm

Hall D

#### **RNA Virus Vectors II**

(Abstracts #396 through #417)

#### **AAV Vectors: Production and Applications**

(Abstracts #418 through #439)

#### **Conditionally Replicating Adenovirus Vectors:**

#### **Adenovirus Toxicity and Immune Response**

(Abstracts #440 through #464)

#### **Lipid Mediated Gene Transfer**

(Abstracts #465 through #487)

#### **Pulmonary Disease**

(Abstracts #488 through #514)

#### **Central Nervous System II**

(Abstracts #515 through #541)

#### **Infectious Diseases and Vaccines**

(Abstracts #542 through #570)

#### **Tumor Vaccines**

(Abstracts #571 through #589)

#### **Cancer Suicide Gene Therapy**

(Abstracts #590 through #607)

#### **Targeted Cancer Therapies II**

(Abstracts #608 through #629)

#### **Gene Regulation: Targets**

(Abstracts #630 through #661)

### **Business Meeting**

4:30 pm – 5:15 pm

Room: 213

### **Corporate Symposium CS340**

7:00 pm – 10:00 pm

Room: 200

#### **HIV-Based Lentiviral Vectors**

*Supported by VIRxSYS*

Lentiviral vectors have shown considerable promise as vectors for efficient and stable gene transfer into human cells. This lentiviral vector symposium brings together the leaders in the field from around the world. The objective of the symposium is to present and discuss the advances in lentivirus-derived vector technology, from the bench to the bedside. The program will begin with a state-of-the-art lecture detailing progress in lentiviral vector applications in general. The program will follow with a series of four presentations that focus on various aspects of current lentiviral vector applications including stem cell gene therapy, infectious disease, RNA-based therapies, and adoptive immunotherapy. The symposium will conclude with a presentation of the most recent data from the first clinical trial evaluating lentiviral vector gene therapy in humans. One should walk away from the symposium with a solid overview of lentiviral vector technology and also with the knowledge of the most recent advances in the field.

*Chair*

Boro Dropulic, PhD

*Speakers*

Inder Verma, PhD - *Lentiviral Vectors: Old and New Uses*  
Luigi Naldini, MD, PhD - *Stem Cell Gene Transfer by Lentiviral Vectors*

Didier Trono, MD - *Interactions between HIV, Lentiviral Vectors, and Target Cells*

John J. Rossi, PhD - *Lentiviral Vector Mediated Delivery of Combinatorial RNA-Based Gene Therapy*

Carl H. June, MD - *Safety and Feasibility of Adoptive Immunotherapy for HIV Infection With Lentiviral Engineered T Cells*

Boro Dropulic, PhD - *Pre-clinical Development of HIV-Based Lentiviral Vectors for a Phase I Clinical Trial for HIV/AIDS*

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Saturday, June 5

#### Scientific Symposia

8:00 am – 10:00 am

##### **SS400 - Cancer: Clinical Cancer Gene Therapy Trials**

Room: Auditorium

*Chair*

Svend O. Freytag, PhD

*Speakers*

Svend O. Freytag, PhD - *Clinical Trials: Combining Oncolytic Viral Therapy and Gene Therapy with Conventional Cancer Therapies*

Robert M. Lorence, MD, PhD - *Phase I Studies of Intravenous Administration of PV701, an Oncolytic Virus*

Alain Rolland, PharmD, PhD - *A Phase 2 Trial of High Dose Allovecin-7 in Patients with Advanced Metastatic Melanoma*

DC Yu, PhD - *Development of Conditionally Replicating Oncolytic Adenovirus for the Treatment of Prostate Cancer: What Have We Learned?*

##### **SS401 - Gene-Based Vaccines: Clinical Applications of Gene-Based Immunotherapeutics and Vaccines-Biodefense Vaccines, Cancer**

Room: 200 A-C

*Chair*

Margaret A. Liu, MD

*Speakers*

Philip R. Johnson, MD - *Recombinant AAV Vectors as Vaccines*

Patrick Squiban, MD - *Treatment of Primary Cutaneous Lymphoma by Interferon Gamma Gene Transfer*

Connie Schmaljohn, PhD - *DNA Vaccines for Hemorrhagic Fever Viruses*

Jeffrey Ulmer, PhD - *Toward the Development of Potent DNA Vaccines for Humans*

##### **SS402 - Neural Disorders: Gene Therapy of Brain Tumors**

Room: 200 D-G

*Chair*

Xandra Breakefield, PhD

*Speakers*

Xandra Breakefield, PhD - *50 Ways to Kill Brain Tumors*

Maria Castro, PhD - *Combined Conditional Cytotoxic and Immune Stimulatory Gene Therapies for Glioblastoma*

Juan Fueyo, MD - *Targeting the Rb Pathway with Oncolytic Adenoviruses: Preclinical Studies*

Seppo Yla-Herttuala, MD, PhD - *Herpes Simplex Virus Thymidine Kinase Gene Transfer in the Treatment of Brain Tumors*

##### **SS403 - Viral: Intracellular Responses to Viral Infection**

Room: 205 A-D

*Chair*

Michael J. Imperiale, PhD

*Speakers*

Robert J. Korst, MD - *Alterations in Dendritic Cell Function Following Adenoviral Infection*

Daniel A. Muruve, MD - *The Molecular Basis of the Innate Response to Viral Vectors*

Jonathan S. Bromberg, MD, PhD - *Innate Immune Responses to Viral Vectors: Modulation by Regulatory T Cells*

Michael G. Katze, PhD - *Will Functional Genomics Unlock the Mysteries of Virus-Host Interactions?*

**Break**

10:00 am – 10:15 am

#### **Simultaneous Oral Presentations**

10:15 am – 12:15 pm

##### **OA410 - Adenovirus Targeting**

Room: 205 A-D

*Moderators*

David T. Curiel, MD, PhD

Robin J. Parks, PhD

(Abstracts #662 - #669)

##### **OA411 – Cancer Tumor Suppressor Gene and Apoptosis**

Room: 208 A-D

*Moderators*

Sunil Chada, PhD

David H. Kirn, MD

(Abstracts #670 - #676)

##### **OA412 – Lipid Mediated Gene Transfer**

Room: Auditorium Room 1

*Moderators*

Ralph Paul, PhD

Volkmar Weissig, PhD

(Abstracts #677 - #684)

##### **OA413 – What's Hot in the Lung: Recent Advances in Lung Gene Therapy**

Room: Auditorium Room 3

*Moderators*

Phillip Factor, DO

Robert Kaner, MD

(Abstracts #685 - #692)

##### **OA414 – Inborn Errors of Metabolism: Lysosomal Storage and Other**

Room: 103 A-C

*Moderators*

Brendan Lee, MD, PhD

Katherine Ponder, MD

(Abstracts #693 - #700)

# The American Society of Gene Therapy's 7th Annual Meeting final program

2004

## Saturday, June 5

### OA415 – Gene Transfer into Hematopoietic Stem/ Progenitor Cells

Room: 102 A-C

*Moderators*

C. Anthony Blau, MD

Jan Nolta, PhD

(Abstracts #701 - #708)

### OA416 – Gene Regulation: Gene Modification and New Methods

Room: 211 A-D

*Moderators*

Brian Johnstone, PhD

Matthew H. Porteus, MD, PhD

(Abstracts #709 - #716)

### Meet-the-Investigator Sessions

12:15 pm – 1:15 pm

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### MI420 - Let's Talk About Site-Specific Integration

Room: L100 A

Michele P. Calos, PhD

### MI421 - Transduction of Sick Hematopoietic Stem Cells

Room: L100 B

Odile Cohen-Haguenauer, MD, PhD

### MI422 - Liver Directed Gene Therapy for Hemophilia and Lysosomal Storage Disease

Room: L100 C

Katherine Ponder, MD

### MI423 - RNAi Based Therapies/Prospects and Problems

Room: L100 D

John J. Rossi, PhD

### MI424 - Assaying and Understanding Vector Insertion and Stem Cell Clonality

Room: L100 E

Christof von Kalle, MD

### Presidential Symposium

1:30 pm – 4:15 pm

Room: Auditorium

*Chair*

Donald B. Kohn, MD

*Speakers*

Gordon Keller, PhD -

*Deriving Tissues from  
Embryonic Stem  
Cells*



Catherine Verfaillie,  
MD - *Unexpected  
Potency of Adult  
Stem Cells: Fact or  
Fiction?*



### Presentation of Top Three Abstracts

#717 – Beverly L. Davidson, PhD – *RNAi Therapy for Dominant Neurodegenerative Diseases*

#718 – Paul Gregorevic, PhD – *Systemic Gene Transfer to Striated Muscles Using rAAV6 Vectors*

#719 – Hildegund Ertl, MD – *E1-Deleted Adenoviral Vectors Persist in Activated Lymphocytes and Cause Continued Transgene Expression and Stimulation of CD8+ Effector T Cells*

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Saturday, June 5

#### Poster Session III

4:00 pm – 7:00 pm

Hall D

#### RNA Virus Vectors III

(Abstracts #720 through #743)

#### AAV Vectors: Genomes and Biology

(Abstracts #744 through #764)

#### Adenovirus Biology and Disease Applications

(Abstracts #765 through #793)

#### DNA Viruses: Other

(Abstracts #794 through #807)

#### Naked DNA-Based Gene Transfer II

(Abstracts #808 through #827)

#### Molecular Conjugates

(Abstracts #828 through #852)

#### Inborn Errors of Metabolism: Lysosomal Storage and Other Diseases

(Abstracts #853 through #879)

#### Bone and Cartilage

(Abstracts #880 through #896)

#### Gene Transfer Applications for Disorders of Hematopoietic Cells

(Abstracts #897 through #923)

#### Cardiac Gene and Cell Therapy

(Abstracts #924 through #940)

#### Cancer Tumor Suppressor Gene and Apoptosis

(Abstracts #941 through #959)

#### Targeted Cancer Therapies III

(Abstracts #960 through #981)

#### Gene Regulation: Vectors

(Abstracts #982 through #1001)

### Sunday, June 6

#### Simultaneous Oral Presentations

9:00 am – 12:00 pm

##### OA500 - Clinical Gene Therapy

Room: 200 A-C

##### Moderators

W. French Anderson, MD

Terence R. Flotte, MD

(Abstracts #1002 - #1009)

##### OA501 - Adenovirus: Toxicity and the Immune Response

Room: 208 A-D

##### Moderators

Andrea Amalfitano, DO, PhD

Maria A. Croyle, PhD

(Abstracts #1010 - #1019)

##### OA502 – Targeting Cancer Therapies

Room: Auditorium Room 1

##### Moderators

R. Michael Blaese, MD

Stephen Russell, MD, PhD

(Abstracts #1020 - #1029)

##### OA503 – Optimization and In Vivo Monitoring of Cancer Suicide Gene Therapy

Room: Auditorium Room 3

##### Moderators

Donald Buchsbaum, PhD

Svend O. Freytag, PhD

(Abstracts #1030 - #1035)

##### OA504 – Naked DNA-Based Gene Transfer

Room: 205 A-D

##### Moderators

David D. Dean, PhD

Todd Giorgio, PhD

(Abstracts #1036 - #1045)

##### OA505 – Gene Regulation: Animal Studies

Room: 103 A-C

##### Moderators

John Forsayeth, PhD

Jeffrey L. Nordstrom, PhD

(Abstracts #1046 - #1055)

##### OA506 – Neuro Gene Therapy II: New Approaches for Dominant, Recessive, Degenerative, and Autoimmune Diseases

Room: 211 A-D

##### Moderators

Thomas Harding, PhD

Deborah Watson, PhD

(Abstracts #1056 - #1064)

Noon – ASGT 7<sup>th</sup> Annual Meeting Concludes

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

#### Vector Integration and Mobilization

Room 205 A-D

##### 1 1:45 PM

*Molecular Long-Term Follow Up in Non-Human Primates That Received Retrovirally Transduced Stem Cells: Despite Proto-Oncogene Insertion No Leukemia or Hematopoietic Abnormalities*

Brian C. Beard, Kirsten A. Keyser, Julie Morris, Bobbie Thomasson, Hans-Peter Kiem

##### 2 2:00 PM

*Promoter Trapping Reveals Significant Differences in Integration Site Selection Between MLV and HIV Vectors*

Michele De Palma, Eugenio Montini, Francesca R. Santoni de Sio, Alessandra Gentile, Enzo Medico, Luigi Naldini

##### 3 2:15 PM

*Effect of Retroviral and Lentiviral Vector Integrations on Transcription of Flanking Genes*

Eugenio Montini, Daniela Cesana, Luigi Naldini

##### 4 2:30 PM

*In Vitro and In Vivo Analysis of Feline Immunodeficiency Virus-Based Lentiviral Vector Integration*

Yubin Kang, Todd E. Scheetz, Christopher J. Moretti, Diane Thi Tran, Litao Xie, Beverly L. Davidson, Thomas L. Casavant, Paul B. McCray, Jr

##### 5 2:45 PM

*Evaluation of the Genotoxic Potential of a Self-Inactivating  $\gamma$ -Globin Lentiviral Vector*

Phillip W. Hargrove, Hideki Hanawa, Steven Kepes, Cheng Cheng, Geoffrey Neale, Arthur W. Nienhuis, Derek A. Persons

##### 6 3:00 PM

*Investigations into the Relative Safety of Lentiviral Vectors to Oncoretroviral Vectors*

Xiaobin Lu, Ziping Chen, Rene Molina, Poorna Ramamurthy, Gwendolyn K. Binder, Boro Dropulic

##### 7 3:15 PM

*The Risk of Integration: Cell Lines for Determining the Frequency and Character of Vector-Mediated Insertional Gene Activation*

Andrea D. Converse, Nikunj V. Somia, R. Scott McIvor

Thursday, June 3

##### 8 3:30 PM

*Integrated Self-Inactivating Lentiviral Vectors Produce Full-Length Genomic Transcripts Competent for Encapsidation and Integration*

Aaron C. Logan, Dennis L. Haas, Tal Kafri, Donald B. Kohn

##### 9 3:45 PM

*Mobilization of Sendai Virus Vectors (SeVV) by Phylogenetic Related Human Pathogens: Implications for Future Vector Generations*

Christian Bernloehr, Sascha Bossow, Sorin Armeanu, Guy Ungerechts, Michael Gregor, Wolfgang J. Neubert, Ulrich M. Lauer, Michael Bitzer

##### 10 4:00 PM

*Cell Cycle-Dependent Transcription from Unintegrated Lentiviral Vector DNA Internal Promoters: Analyses with Class I Integrase Mutants*

Dyana Saenz, Nils Loewen, David Leske, Kyle Howell, Jonathan Holmes, Margaret Good, Eric Poeschla

### AAV Vectors: Genome and Capsid Modifications

Auditorium Room 1

##### 11 1:45 PM

*Reporter Gene-Based Analysis of rAAV Genome Circularization and Recombination*

Douglas M. McCarty, Vivian Choi, Richard J. Samulski

##### 12 2:00 PM

*A New Class of Hybrid Adeno-Associated Viral Vectors with Non-Homologous ITRs Improves Directional Recombination and Dual-Vector Reconstitution of Large Transgenes*

Ziying Yan, Roman Zak, Yulong Zhang, John F. Engelhardt

##### 13 2:15 PM

*Hot Spots for rAAV2 Vector Integration in Mice*

Hiroyuki Nakai, Xiaolin Wu, Sally Fuess, Theresa Storm, Shawn Burgess, Markus Grompe, Mark A. Kay

##### 14 2:30 PM

*Absence of T-Shaped Structure and Deletions of B and C Hairpins Have Minimal Effects on Essential Functions of AAV Inverted Terminal Repeats*

Xiaodong Zhu, Juan Li, Xiao Xiao

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

**15 2:45 PM**

*Trans-Splicing Adeno-Associated Viral Vector-Mediated Gene Therapy Is Limited by Transcription Across the ITR Junction but Not by Dual Vector Co-Infection Efficiency*

Zhuping Xu, Yongping Yue, Chaoyang Ye, Jianming Qiu, David J. Pintel, Dongsheng Duan

**16 3:00 PM**

*Visualization of the Foci of Recombinant AAV Replication and Their Relationship with Cellular Proteins Involved in the Recognition and Repair of Double-Stranded DNA Breaks*

Tiziana Cervelli, Lorena Zentilin, Alessandro Marcello, Mauro Giacca

**17 3:15 PM**

*Role of Vp2 in AAV Packaging*

Joshua C. Grieger, Joseph E. Rabinowitz, Dawn E. Bowles, Richard J. Samulski

**18 3:30 PM**

*Incorporation of the Green Fluorescent Protein Into the Adeno-Associated Virus Type 2 Capsid*  
Kerstin Lux, Nico Goerlitz, Kristin Leike, Daniela Goldnau, Stefan Finke, Michael Hallek, Hildegard Buening

**19 3:45 PM**

*The Transcapsidation of AAV Serotypes*

Joseph E. Rabinowitz, Dawn E. Bowles, Susan M. Faust, Julie G. Ledford, Scott E. Cunningham, R. Jude Samulski

**20 4:00 PM**

*Directed Evolution of AAV2 Yields Novel Capsid Variants*

Narendra Maheshri, Brian Kaspar, David V. Schaffer

### Novel Cancer Targeted Therapeutics

Room 208 A-D

**21 1:45 PM**

*Induction of CEA in Pulmonary Vasculature to Study Targeting Strategies for Cancer*

Maaike Everts, SangAe Kim-Park, Long P. Le, Paul N. Reynolds, William E. Grizzle, David T. Curiel

**22 2:00 PM**

*Tumor Homing of Genetically Modified Bone Marrow Cells*

Zong-Yi Li, Shaoheng Ni, Tobias Neff, Dmitry Shayakhmetov, Andre Lieber

**Thursday, June 3**

**23 2:15 PM**

*T Cell Carriers for Gene Delivery to Tumors*

Caroline Cole, Rosa M. Diaz, Michael Forshaw, Timothy Kottke, Jill Thompson, Michael Gough, Xandra Breakefield, Miguel Sena-Esteves, Jian Qiao, Richard G. Vile

**24 2:30 PM**

*Antibody-Targeted Fusion Provides a Versatile Platform for Novel Targeted Therapies*

Takafumi Nakamura, Kah-Whye Peng, Sompong Vongpunsawad, Mary Harvey, Hiroyuki Mizuguchi, Takao Hayakawa, Roberto Cattaneo, Stephen J. Russell

**25 2:45 PM**

*Molecular Imaging-Guided Gene Therapy of Experimental Gliomas*

Maria A. Rueger, Alexandra Winkeler, Lisa Li, Benedikt Rueckriem, Stefan Vollmar, Wolf D. Heiss, Andreas H. Jacobs

**26 3:00 PM**

*Tropism Illustrated: How a Morbillivirus Takes over the Immune System*

von Messling Veronika, Milosevic Dragana, Cattaneo Roberto

**27 3:15 PM**

*Differential Phage Display for Discovery of Nuclear Localization Peptides: Application to Mammary Adenocarcinoma*

Ashley A. Weiner, Todd D. Giorgio

**28 3:30 PM**

*A Clinically Relevant Ex Vivo Toxicity Assay Using Human Liver Slices To Determine Specificity of Oncolytic Viruses*

Marianne G. Rots, Marieke GL Elferink, Dorenda Oosterhuis, Peter Olinga, David T. Curiel, Hidde J. Haisma, Geny M. M. Groothuis

**29 3:45 PM**

*In Vivo Imaging and Therapy of Breast Cancer Xenografts Following MUC1 Promoter-Driven Expression of the Sodium Iodide Symporter (NIS)*

Roisin M. Dwyer, Elizabeth R. Bergert, Michael K. O'Connor, Sandra J. Gendler, John C. Morris

**30 4:00 PM**

*Patient Oriented DNA Based Therapy from the Bench to the Patient*

Patricia Ohana, Ofer Gofrit, Basem Ayesh, Imad Matouk, Tatiana Birman, Suhail Ayesh, Aya Mizrahi, Ami Sidi, Tamar Schneider, Nathan de Groot, Abraham Hochberg

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Thursday, June 3

#### Inborn Errors of Metabolism: Hemophilia and Other Disorders

Auditorium Room 3

**31 1:45 PM**

*Creation of a Hemophilia B Mouse Expressing Defective Human Factor IX*

Paul E. Monahan, Tai-Ping Zhang, Da-Yun Jin, Tong Gui, Darrel W. Stafford

**32 2:00 PM**

*Efficacy and Safety of Mouse Coagulation Factor VIIa Gene Transfer in Hemophilia B Mice*

Majed N. Aljamali, Paris Margaritis, Valder R. Arruda, Alexander Schlachterman, Rodney M. Camire, Katherine A. High

**33 2:15 PM**

*Induction of Antigen-Specific T Cell Anergy and Deletion by Hepatic AAV Gene Transfer*

Eric Dobrynski, Lixin Wang, Ou Cao, Federico Mingozi, Roland W. Herzog

**34 2:30 PM**

*Retroviral Vector-Mediated Neonatal Gene Therapy Results in Long-Term and Therapeutic Levels of Canine Factor VIII Expression in Hemophilia A Mice and Dogs*

Lingfei Xu, Timothy C. Nichols, Rita Sarkar, Stephanie McCorquodale, Dwight Bellinger, Katherine P. Ponder

**35 2:45 PM**

*Downregulation of Immune Responses Induced by Oral DNA Administration*

Andrew Gomez Vargas, Gonzalo Hortalano

**36 3:00 PM**

*Permanent Phenotypic Correction of Haemophilia B in Immunocompetent Mice by Prenatal Gene Therapy*

Simon N. Waddington, Megha S. Nivsarkar, Ajay R. Mistry, Suzanne M. K. Buckley, Geoffrey Kemball-Cook, Karen L. Mosley, Kyriacos Mitrophanous, Pippa Radcliffe, Maxine V. Holder, Mairi Brittan, Tassos Georgiadis, Faisal Al-Allaf, Brian W. Bigger, Lisa G. Gregory, Terence H. Cook, Robin R. Ali, Adrian Thrasher, Edward G. D. Tuddenham, Mike Themis, Charles Coutelle

**37 3:15 PM**

*Duration of Immunologically Detectable AAV-2 Capsid in an Animal Model*

Denise E. Sabatino, Federico Mingozi, David C. Brooks, Alex Schlachterman, Shyrie Edmonson, Linda Couto, Peter Colosi, Haifeng Chen, Hildegund C. J. Ertl, Katherine A. High

**38 3:30 PM**

*AAV-Mediated Gene Therapy for Systemic Lupus Erythematosus (SLE)*

Xiaojing Ye, Tong Zhu, Juan Li, Xiao Xiao

**39 3:45 PM**

*Reversal of Diabetes by Inducing Islet Neogenesis Using Helper Dependent Adenovirus Mediated Gene Therapy*

Vijay Yechoor, Hideto Kojima, Mineko Fujimiya, Bonne Thompson, Benny Chang, Laura Nelly, Philip Ng, Lawrence Chan

**40 4:00 PM**

*Correction of Dyslipidemia in Murine and Feline Models of Lipoprotein Lipase Deficiency by Intramuscular Administration of AAV1-LPLS447X*

Colin J. D. Ross, Jaap Twisk, Jan Albert Kuivenhoven, Guoqing Liu, Fudan Miao, Karen van den Oever, Maarten Beetz, Dennis Verbart, Andrew Bakker, Paul P. A. Oranje, Wim T. Hermens, Paul Dijkhuizen, John J. P. Kastelein, Janneke J. M. Meulenbergh, Michael R. Hayden

#### Neuro Gene Therapy I: New Approaches for Dominant, Recessive, Degenerative, and Autoimmune Diseases

Room 103 A-C

**41 1:45 PM**

*Ex Vivo Gene Therapy Provides a Unique Therapeutic Benefit and Corrects Metachromatic (MLD) and Globoid (GLD) Leukodystrophy in Mouse Models*

Alessandra Biffi, Ilaria Visigalli, Angelo Quattrini, Ilaria Bertani, Michele De Palma, Ubaldo Del Carro, Riccardo Brambilla, Sergio Marchesini, Claudio Bordignon, Luigi Naldini

**42 2:00 PM**

*AAV-Mediated Gene Therapy Causes Functional Improvements in a Murine Model of Infantile Neuronal Ceroid Lipofuscinosis (INCL, Batten Disease)*

M. Griffey, D. Wozniak, M. Wong, S. Rothman, E. Bible, J. Cooper, C. Vogler, M. Sands

**43 2:15 PM**

*Immunological Response Characterization in an Alzheimer's Disease Mouse Model Following HSV Amplicon-Based A $\beta$  Vaccination*

William J. Bowers, Michael A. Mastrangelo, Hilary A. Stanley, Ya-Hui Chiu, Howard J. Federoff

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

**44 2:30 PM**

*AAV Retrograde Transport Potential and Therapeutic Approaches for ALS*

Brian K. Kaspar, Jeronia Vich, Lindsey Christian, Jeffrey D. Rothsten, Fred H. Gage

**45 2:45 PM**

*Adenoviral Tetanus Toxin Light Chain Gene Expression in the Brainstem Induces Specific Motor Inhibition*

Ajit A. Krishnaney, Zuowei Zhao, Qingshang Teng, James Liu, Diana Tenase, Mary Garrity-Moses, Michael Davis, Nicholas M. Boulis

**46 3:00 PM**

*Clinical and Functional Recovery from Experimental Autoimmune Encephalomyelitis by Intracisternal Delivery of IL4 from a Helper-Dependent Adenoviral Vector: A Pre-Clinical Model of Gene Therapy of Multiple Sclerosis*

Erica Butti, Alessandra Recchia, Alessandra Bergami, Anna Stornaiuolo, Elena Brambilla, Roberto Furlan, Gianvito Martino, Fulvio Mavilio

**47 3:15 PM**

*Cytokine Gene-Modified Mesenchymal Stem Cells Promote Functional Recovery and Reduce Infarct Size in the Rat Middle Cerebral Artery Occlusion Model*

Kazuhiko Kurozumi, Kiminori Nakamura, Tomotsugu Ichikawa, Yasuhiro Ono, Takashi Tamiya, Yoshinori Ito, Osamu Honmou, Kiyohiro Houkin, Hirofumi Hamada, Isao Date

**48 3:30 PM**

*In Vivo and In Situ Delivery of Huntingtin-Specific Single-Chain Fv Intrabody Genes as Therapeutic Agents for Huntington's Disease*

Todd W. Miller, William J. Wolfgang, Kevin Manley, Yangsheng Zhou, Chun Zhou, Jack M. Webster, Nicholas D. Mazarakis, Anne Messer

**49 3:45 PM**

*AAV Vector-Mediated Gene Delivery of Aromatic L-Amino Acid Decarboxylase Restored L-DOPA Efficacy in a Primate Bilateral Model of Parkinson's Disease*

Shin-ichi Muramatsu, Fumiko Ono, Yuko Nara, Mika Kodera, Naomi Takino, Junko Tsuchida, Katsuyoshi Kawasaki, Kunihiko Ikeguchi, Kenichi Fujimoto, Hideo Tsukada, Keiji Terao, Imaharu Nakano, Keiya Ozawa

**50 4:00 PM**

*AAV-Mediated Expression and Secretion of ADNF-9 Protects Substantia Nigra Neurons from 6-OHDA Damage*

Sophia Papadeas, George R. Breese, Thomas J. McCown

**Thursday, June 3**

### Gene Regulation: Stem Cells

Room 102 A-C

**51 1:45 PM**

*A Novel Approach to Stem Cell Gene Therapy: Controlling Stem Cell Differentiation with Engineered Zinc-Finger Protein Transcription Factors*

Victor V. Bartsevich, Jeffrey C. Miller, Casey C. Case, Carl O. Pabo

**52 2:00 PM**

*Gut Enriched Kruppel-Like Factor (GKLF) Inhibits Differentiation and Induces Self-Renewal of Murine Embryonic Stem Cells*

Rebecca J. Chan, Yanjun Li, Li Zhong, Mervin C. Yoder

**53 2:15 PM**

*Inducible FGFr-1 Stimulates Self Renewal of Hematopoietic Cells*

Michael A. Weinreich, C. Anthony Blau

**54 2:30 PM**

*Restriction of Transgene Expression to the B-Lymphoid Progeny of Human Lentivirally Transduced CD34+ Cells*

Thomas Moreau, Florence Bardin, Jean Imbert, Christian Chabannon, Cecile Tonnelle

**55 2:45 PM**

*Alteration of Primitive Hematopoietic Cell Fate and Growth by the GATA-2 Transcription Factor*

Stacy L. Throm, Derek A. Persons

### Adenovirus Biology and Disease Applications

Room 211 A-D

**56 1:45 PM**

*Intraplacental Gene Transfer of IGF-1 Corrects Intrauterine Growth Restriction in the Rabbit Model*

Sundeep G. Keswani, Anna B. Katz, Elliot B. Kozin, Phillip W. Zoltic, Timothy M. Cromblehome

**57 2:00 PM**

*Persistent PEDF Expression Following Intravitreal Administration of High-Capacity Adenovectors*

Joseph T. Bruder, Ping Chen, Kurt Kroeger, Melissa Hamilton, Miguel Carrion, Douglas E. Brough, Lisa L. Wei, C. Richter King, Peter A. Campochiaro, Peter Gehlbach

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Thursday, June 3

**58 2:15 PM**

*Toward Adenoviral Mediated RNA Interference for the Treatment of Hepatitis B Virus Infection*  
Anton P. McCaffrey, Kusum Pandey, Anja Ehrhardt, Zan Huang, Mark A. Kay

**59 2:30 PM**

*Development of Conditionally Replicative Adenoviruses as Platforms for the Expression of shRNAs or Therapeutic Genes*  
Jan E. Carette, Frederik H. E. Schagen, Renée M. Overmeer, Jeroen D. C. Mastenbroek, Winald R. Gerritsen, Victor W. van Beusechem

**60 2:45 PM**

*Site-Specific Integration in Human Somatic Cell DNA by Adeno/AAV Hybrid Vectors*  
Alessandra Recchia, Laura Perani, Daniela Sartori, Clelia Olgiati, Fulvio Mavilio

### Other DNA Viruses

Room 211 A-D

**61 3:00 PM**

*Prevention of Liver Cirrhosis Development in Rats with Recombinant SV40 Vectors Expressing IGF-I*  
Maria Vera, Laura Martinez, Mikel Zaratiegui, Carlos M. Rodriguez, Jesus Prieto, Puri Fortes

**62 3:15 PM**

*Ras Signaling as a Marker for Application of Oncolytic Herpes Mutant Against Lymphoma*  
Faris Farassati, Natasha Atanaskova, Corey Raffel, Patrick Johnston, Patrick Lee

**63 3:30 PM**

*Re-Directed Herpes Simplex Virus Infection by a Soluble, Bi-Specific Adapter Protein for gD in Combination with Nectin-1 Blocking Agents*  
Kenji Nakano, Ryutaro Asano, Kohei Tsumoto, William F. Goin, Izumi Kumagai, Justus B. Cohen, Joseph C. Glorioso

**64 3:45 PM**

*Bioluminescence Imaging of Regulated Gene Expression Using HSV-1 Amplicon Vectors in Rodent Brain*  
Sam Wang, Jeremy Petracic, Xandra O. Breakefield

**65 4:00 PM**

*Systemic Cancer Therapy with an Enhanced Tumor-Selective Vaccinia Virus Lacking Anti-Apoptosis Viral Genes Spi-2 and Spi-1*  
Zongsheng Guo, Arpana Naik, Yun Hu, Richard DeMarco, Mark O'Malley, Richard Alexander, Bernard Moss, Mike Lotze, David L. Bartlett

### Poster Session I

Hall D

### **RNA Virus Vectors I**

**66**

*A Safe and Efficient Lentiviral Vector System: Optimization of Virus Production and Purification*  
Donald S. Anson

**67**

*Bovine Immunodeficiency Virus Based Lentiviral Vector with Improved Safety Features*  
Rene P. Molina, Hongqiang Q. Ye, James Brady, Jingli Zhang, Heather Zimmerman, Michael Kaleko, Tianci Luo

**68**

*Improved Lentiviral Vector System with Enhanced Safety Features*  
Jill Oldenburg, Aaron C. Logan, Maria Barcova, Celina Ngiam, Paula M. Cannon

**69**

*Closing-In on the Titer Gap Between Transient Transfection and Packaging Cell Lines*  
Adam S. Cockrell, Kailing Fu, Hong Ma, Thomas McCown, Tal Kafri

**70**

*Stable Packaging Cell Lines Development for the Production of Lentiviral Vectors*  
Sophie Broussau, Nadine Jabbour, Guillaume Lachapelle, Yan Xu, Alaka Mullick, Renald Gilbert, Bernard Massie

**71**

*Chimeric Lentiviral Vectors Derived from HIV-1 and HIV-2*  
Suresh K. Arya, Jenice D'Costa, Geetanjali Sachdeva, Jean Cho, Kritika Kachapati

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Thursday, June 3

**72**

*Position and Copy Number Dependence of the Central Polypurine Tract (cPPT) Function in Simple and Complex Lentiviral Vectors*

Alex H. Chang, Leszek Lisowski, Matthias Stephan, Isabelle Rivière, Michel Sadelain

**73**

*An Inhibitory Effect of a Sequence Just Upstream of the Central Polypurine Tract of HIV-1 on RNA Production of an HIV-1-Based Vector*

Ryuta Sakuma, Aikichi Iwamoto, Yoshihiro Kitamura

**74**

*Factors Affecting Long Terminal Repeat (LTR)-Driven Gene Expression Using VL30 Retro-Vectors and DNA Plasmid Vectors*

Jim Williams, Jeremy Luke, Clague Hodgson

**75**

*The Optimization of a Single-LTR HIV-1 Vectors for Functional Genomic Applications*

Hong Ma, Tal Kafri

**76**

*Development of Multigene Lentiviral Vectors Containing the Encephalomyocarditis Virus IRES and Foot-and-Mouth Disease Virus 2A Cleavage Factor*

Dhanalakshmi Chinnasamy, Michael Milsom, James Neuenfeldt, Geoffrey P. Margison, Leslie J. Fairbairn, Nachimuthu Chinnasamy

**77**

*Production and Purification of Measles Virus for Oncolytic Virotherapy Clinical Trials*

Kirsten K. Langfield, Troy R. Wegman, Henry J. Walker, Guy E. Griesmann, Julie A. Sauer, Sharon A. Stephan, Mark J. Federspiel

**78**

*Enhancement of Retroviral-Mediated Gene Transfer by Purification of Virus Stocks from Inhibitors of Transduction*

Natalia Landazuri, Joseph M. Le Doux

**79**

*Validation of Viable Producer Cell Removal/Inactivation from Clinical-Grade Oncoretroviral Vector Supernatant Produced in Cell Factories*

Mark Przybylowski, Amir Hakakha, Yong Fan, Isabelle Riviere

**80**

*A Rapid and Effective Lentivirus Purification Procedure*

Christine R. Rowley, Patrick L. Sinn, Linda Bullinga, Paul

B. McCray, Jr, Beverly L. Davidson

**81**

*Modification of HEK 293 Cell Integrin Expression Profile Allows Convenient Large-Scale Roller Bottle Production of Lentiviral Vectors*

Manij Patel, David A. Wilcox, Angela M. Giddings, Tristan R. McKay, John C. Olsen

**82**

*Adaptation of a Lentiviral Vector Producer Cell Line to Reduced-Serum, Suspension Culture for Large Scale Production*

Kay Townsend, Yong Chen, Jonathan Dayao, Deborah Farson, William M. Miller, Andy Lin

**83**

*Development of an RCL Assay for ElAV Based Products*

James E. Miskin, Holger Roehl, Denise Westfall, Jonathan Rohll, Daniel Chipchase, Douglas Jolly, Susan M. Kingsman, Kyriacos A. Mitrophanous

**84**

*Enhanced Detection Method for Retrovirus in Vaccine*

Ji Young Yoo, Beom-Seok Suh, Sue Nie Park, Joo-Hang Kim, Chae-Ok Yun

**85**

*High-Level Expression of Wild-Type p53 from the Novel p53-Responsive pCLPG Retrovirus Halts the Proliferation of Tumor Cells Regardless of Their p53 Status*

Bryan E. Strauss, Marcio C. Bajgelman, Eugenia Costanzi-Strauss

**86**

*Mutation Frequency of the p53 cDNA Introduced by the Retroviral Vector*

Su Su, Atushi Watanabe, Takashi Shimada

**87**

*APOBEC3G and Immunity to Retroviruses*

Reuben S. Harris

### Serotypes Capsids and Specific Applications

**88**

*AAV Clades: Their Ability To Recombine and Cross Species-BARRIERS*

Luk H. Vandenberghe, Guangping Gao, Mauricio R. Alvira, Roberto Calcedo, James M. Wilson

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Thursday, June 3

**89**

*Isolation of a Close AAV5 Relative from Goat Tissues: Evidence of Host Promiscuity*  
Alejandra E. Arbetman, Michael A. Lochrie, Britta Randlev, Richard T. Surosky, Shangzhen Zhou, Jennifer Wellman, Cheryl Pater, Howard Lehmkuhl, Lea Ann Hobbs, Glenn Pierce, Peter Colosi

**90**

*Evaluation of Novel Gene Transfer Vectors Derived from Infectious Molecular Clones of Primate AAVs*  
Xiaoyang Zhou, Guangping Gao, Roland A. Owens, Roberto Calcedo, James M. Wilson

**91**

*Universal Purification of AAV Serotypes 1-5 Modified To Contain a Heparin Binding Epitope*  
Susan M. Faust, Dawn E. Bowles, Scott E. Cunningham, Joseph E. Rabinowitz, R. Jude Samulski

**92**

*Metabolically Biotinylated Adeno-Associated Virus (AAV) Types 1, 2, 3, 4 and 5 for Scalable Purification and Vector Retargeting*  
A. Kate Sasser, Gregory S. Arnold, Jeffrey S. Bartlett

**93**

*Targeting AAV to Brain Vascular Endothelium*  
Yong Hong Chen, Beverly L. Davidson

**94**

*Targeting Endothelial Cells in Mice with Adeno-Associated Virus Serotype 1 Vector*  
Haiyan Fu, Joseph Muenzer, Richard J. Samulski, Kang Lu, Jerillyn S. Jennings, Douglas M. McCarty

**95**

*Retargeting AAV-2 to Angiogenic Endothelial Cells with Tumor Vasculature Specific Capsid Variants*  
Andreas Riederer, Bernard Kaess, Luca Perabo, Hildegard Buening, Michael Hallek

**96**

*Transgene Expression from Recombinant AAV-8 Vectors in the Liver Occurs Independently of the Serotype Origin of the Viral Inverted Terminal Repeats*  
Dirk Grimm, Hiroyuki Nakai, Theresa A. Storm, Efren P. Riu, Mark A. Kay

**97**

*Selection of Potent AAV Pseudotypes To Transduce Rat Pancreatic Islets*  
Brigitte Le Mauff, Françoise Boeffard, Nasro Benrezzak, Ignacio Anegon

**98**

*Adeno-Associated Virus Serotype 8 as a Vector for HEPATIC Delivery of alpha-1 Anti-Trypsin (AAT)*  
Thomas J. Conlon, Travis L. Cossette, Terence R. Flotte

**99**

*Long-Term Expression of Soluble TNFR:Fc Protein Following Multiple Administrations of AAV-TNFR:Fc Vector Pseudotyped with Capsids of Alternate Serotypes*  
Ziv Sandalon, Kurt Lustig, Haim Burstein

**100**

*An Evaluation of the Use of Adeno-Associated Virus Serotypes AAV1 and AAV8 To Obtain Stable Therapeutic Levels of Apolipoprotein A-I in Mouse Plasma*  
Karen F. Kozarsky, Deborah A. Welham, Mary H. Donahee

**101**

*Toxicology and Biodistribution Studies of a Recombinant Adeno-Associated Virus 2 (rAAV2) Alpha-1 Antitrypsin (AAT) Vector*  
Amy E. Poirier, Lynn A. Combee, Ashley T. Martino, Terence R. Flotte

**102**

*Catheter-Based Intrahepatic rAAV Gene Delivery: Influence of Infusion Flow Rate and Volume on Uptake*  
Lin He, Dea Nagy, Joe Vargas, Tongyao Liu, Ciaran Scallan, Shangzhen Zhou, Sharmila Vijay, Jurg Sommer, Kirk W. Johnson, Linda B. Couto, Pierce F. Glenn, Bin Yang

**103**

*Comparison of the Efficacy on Gene Transfer by AAV Vectors Delivered by Distinct Routes of Administration to the Liver of Hemophilia B Dogs*  
Valder Arruda, Ciaran Scallan, Haiyan Jiang, Linda Couto, Roland W. Herzog, Timothy Nichols, Glenn Pierce, Katherine A. High

**104**

*A Novel Deletion in the FVIII B-Domain That Reduces Transgene Size While Preserving FVIII Activity*  
Yi-Lin Liu, Hua Zhu, Alexander Schlachterman, Heesoon Chang, Rodney M. Carime, Katherine A. High

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Thursday, June 3

**105**

*Optimization of Short-Hairpin RNAs for the Treatment of the Hepatitis C Virus Using AAV Vectors with Multiple Expression Cassettes*

Peter W. Roelvink, David A. Suhy, Alexander A. Kolykhalov, Sara M. Cunningham, Taka Mizutani, Mark A. Kay

**106**

*Functional Human alpha 1 Antitrypsin Secreted from Muscle Transduced by Adeno-Associated Virus (rAAV7) Vector*

Yuanqing Lu, Martha Campbell-Yhompson, Chengwen Li, Terence R. Flotte, Sihong Song

**107**

*Use of Adeno-Associated Viral Vectors for Effective Delivery of Engineered Zinc Finger Protein Transcription Factors*

Xuxin Liang, Linda Hinh, Steve Zhang, Lei Zhang, Deng-Feng Xia, Kristine Venstrom, S. Kaye Spratt, Edward Rebar, Casey Case

**108**

*In Vitro Down-Regulation of Transgene Expression with Cre/loxP Mediated Recombination*

J. Lawrence Merritt II, Jan Daniels, David B. Schowalter

**109**

*Cyclical Mechanical Stretch and AAV2 Mediated Gene Transfer to Cultured Human Vascular Cells*

Sabyasachi Sen, Jeff S. Bartlett, Yvonne Birney, Paul Cahill, Timothy O'Brien

### Adenovirus Targeting

**110**

*Helper-Dependent Adenovirus with the Cytokeratin-18 Promoter Produces Greater Transepithelial Chloride Current and More Persistent Transgene Expression Than the CMV Promoter in Differentiated Human Airway Epithelia*

Sara L. Farmen, Philip H. Karp, Philip Ng, David R. Koehler, Jim Hu, Joseph Zabner, Michael J. Welsh

**111**

*A Chimeric Adenovirus Vector Encoding Reovirus Attachment Protein Sigma 1 Targets Cells Expressing Junctional Adhesion Molecule 1 and Sialic Acid*

George T. Mercier, Jacquelyn A. Campbell, James D. Chappell, Terence S. Dermody, Thilo Stehle, Michael A. Barry

**112**

*Gene Transfer into a Hematopoietic Cell Line Using a Helper-Dependent Adenoviral Vector with the Chimeric Ad5/35 Fiber*

Michael A. Balamotis, Katie Huang, Ko Mitani

**113**

*Role of CD46 on Ad35 Vector-Mediated Transduction*

Fuminori Sakurai, Hiroyuki Mizuguchi, Kenji Kawabata, Naokazu Inoue, Masaru Okabe, Teruhide Yamaguchi, Takao Hayakawa

**114**

*Adenoviral Vectors for Gene Therapy with Improved Entry of Malignant Glioma*

Eric Brouwer, Olga J. Ophorst, Menzo J. E. Havenga, Bertie H. C. G. M. de Leeuw, Rob C. Hoeben, Peter A. E. Silleveld Smitt

**115**

*Genetic Fiber Mosaic Adenovirus Confers New Properties to Adenoviral Particles*

Larisa Pereboeva, Svetlana Komarova, David T. Curiel

**116**

*Members of Adenovirus Subgroup B Utilize CD80 (B7.1) and CD86 (B7.2) as Primary Cellular Attachment Receptors*

Joshua J. Short, Alexander V. Pereboev, Yosuke Kawakami, Chenthamarakshan Vasu, Mark J. Holterman, David T. Curiel

**117**

*Antitumor Immunity Induced by Adenovirus Vector-Mediated Transduction of OX40 Ligand*

Toshiaki Kikuchi, Sita Andarini, Toshihiro Nukiwa

**118**

*Efficient Infection of Head and Neck Cancer Cells by Ad5/35 Hybrid Virus*

Elina Suominen, Reidar Grenman, Mikko Savontaus

**119**

*Studies on the Targeting of Adenoviral Vectors Carrying a Tumor-Cell Specific Peptide*

Karola Rittner, Valerie Schreiber, Klaus Schughart, Nicolas Futin, Catherine Ledoux, Monika Lusky

**120**

*Transient Saturation of the Reticuloendothelial System by Non-Cytotoxic Liposomes Increases Hepatocyte Transduction After Adenoviral Gene Transfer*

Jan Snoeys, Geertrui Mertens, Joke Lievens, Désiré Collen, Eric Biessen, Bart De Geest

# The American Society of Gene Therapy's 7th Annual Meeting final program

# 2004

## Abstract Directory

Thursday, June 3

121

*Specific Targeting of Adenoviral Vectors Using the Reovirus Attachment Protein*

Frederik H. E. Schagen, Jan E. Carette, Terence S. Dermody, Winald R. Gerritsen, Victor W. van Beusechem

122

*Expression of hCD40 in the Pulmonary Endothelium Targeted Via a Fiber-Modified Adenovirus Improves Gene Delivery to the Lung*

Miiru Izumi, Yosuke Kawakami, Joel N. Glasgow, Natalya Belousova, David T. Curiel

123

*Adenovirus Type 11 Based Vectors as a Platform for Treatment of Metastatic and Hematopoietic Disease*

Daniel Stone, Andre Lieber

124

*Efficient Transduction and Activation of Dendritic Cells by Adenoviral Vectors Targeted to DC-SIGN*

Nikolay Korokhov, Wayne A. Aldrich, Alex Krendelchitchikov, Angela Granelli-Piperno, Papia T. Banerjee, Stephen D. Gillies, Pierre L. Triozzi, Abner M. Mhashilkar, David T. Curiel

125

*Formulation and In Vitro Properties of Adenovirus-Polylactide Nanoparticle Affinity-Based and Magnetic Composites*

Michael Chorny, Ilia Fishbein, Ivan S. Alferiev, Origene Nyanguile, Robert J. Levy

126

*Zc-Ad Vector: A Novel Immunotargeting Vector with a Modified Fc-Binding Domain*

Sam C. Noureddini, Nikolay Korokhov, Abner Mhashilkar, Joanne T. Douglas, David T. Curiel

127

*Adenovirus Type 5 Vectors Pseudotyped with Fibers from Subgroup D (19p and 37) Show Detargeting from Hepatocytes In Vitro and In Vivo*

Laura Denby, Delyth Graham, Dan J. Von Seggern, Stuart A. Nicklin, Andrew H. Baker

128

*Use of Chimeric Adenoviral Vectors To Assess Capsid Neutralization Determinants*

Soumitra Roy, David Clawson, Corinna Lebherz, Roberto Calcedo, Julio Sanmiguel, Di Wu, James M. Wilson

129

*Infectivity Enhancement of an Adenovirus Vector Via Genetic Incorporation of the Reovirus Spike Protein Sigma 1*

Yuko Tsuruta, Larisa Pereboeva, Joel N. Glasgow, Cindy L. Luongo, Svetlana Komarova, Yosuke Kawakami, David T. Curiel

130

*Targeting of Adenovirus Vectors to Mesothelin Via a Bispecific Antibody Reveals Enhanced Gene Transfer to Human Pancreatic Cancer Cells*

Michael A. van Geer, Ira Pastan, Victor W. van Beusechem, Hidde J. Haisma, Piter J. Bosma, Dirk J. Gouma, John G. Wesseling

131

*Cell-Targeting Ligand Discovery Using Context-Specific Phage-Presenting Peptide Libraries*

Debadyuti Ghosh, Michael A. Barry

132

*Lentivirus-Mediated Expression of Adenovirus Fibers for the Complementation or Packaging of Fiber Gene-Mutated or -Deleted Adenovirus Vectors*

Taco G. Uil, Jort Vellinga, On Ying Chan, Stephanie van der Heijdt, Rob Hoeben

133

*Enhanced Expression of ABCA4 Stargardt Protein Mediated by Adenoviral Vectors in a Simulated Ocular Environment*

Joshua N. Mallam, Mary Y. Hurwitz, Donna Palmer, Philip Ng, Richard L. Hurwitz

134

*Structural Requirements of Adenovirus Targeting Via by Modification of Minor Capsid Protein IX*

Rob C. Hoeben, Steve J. Cramer, Leif Lindholm, Martijn J. Rabelink, Diana J. Van den Wollenberg, Stephanie Van der Heijdt, Jort Vellinga

135

*A New Ligand-Pseudoreceptor System for the Generation of Adenoviral Vectors with Altered Tropism*

Yue Zeng, Jairo Jaime, Lucie Bourget, Dreg D. Crescenzo, Maureen O'Connor, Bernard Massie

136

*Vector Host Interactions of Group B Adenoviruses in CD46 Transgenic Mice*

Anuj Gaggar, Dmitry M. Shayakhmetov, Sonia Marwah, Shaoheng Ni, Andre M. Lieber

# The American Society of Gene Therapy's 7th Annual Meeting final program

2004

## Abstract Directory

Thursday, June 3

137

*pFex: A Novel Fiber Exchange System for the Creation of Modified-Fiber Gene Therapy Vectors*  
Shawn E. Lupold, Wasim H. Chowdhury, Ronald Rodriguez

### Naked DNA-Based Gene Transfer I

138

*Purification of Plasmid DNA on an Anion Exchange Column Without the Use of RNase To Process Scale*  
Tina M. Pitarresi, Sibylle U. Herzer, Bob Kennedy, Peter Moore

139

*Optimization of Electroporation Parameters for the Intramuscular Delivery of Plasmids in Pigs*  
Amir S. Khan, Louis C. Smith, Ronald V. Abruzzese, Kathleen K. Cummings, Melissa A. Pope, Patricia A. Brown, Ruxandra Draghia-Akli

140

*Hydrodynamics-Based Transfection of the Liver: Entrance into Hepatocytes of DNA That Causes Expression, Takes Place Very Early after Injection*  
Fanjam Andrianaivo, Michèle Lecocq, Simone Wattiaux-De Coninck, Robert Wattiaux, Michel Jadot

141

*Development of a One-Step Column Purification of Minicircle Vector DNA Devoid of Bacterial Sequences That Results in High Level Persistent Transgene Expression In Vivo*  
Zhi-Ying Chen, Cheng-Yi He, Mark A. Kay

142

*Innovations in Process Technology for Plasmid DNA Manufacturing – Modular Variations of a Generic Process, Regulatory Relevance, and Economic Impact*  
Wayne A. Tvrlik, John Vu, Astrid Breul, Markus Mueller, Joachim Schorr

143

*Use of Ciliated Cell Specific Promoter FoxJ1 in Gene Transfer Vectors for the Airway Epithelium*  
Anna E. Lawton, Stephanie G. Sumner-Jones, Anusha Varathalingam, Lee A. Davies, Stephen C. Hyde, Deborah R. Gill

144

*Duration of Reporter Gene Expression from Naked pDNA in the Mouse Lung Following Direct Electroporation and Development or Wire Electrodes for Sheep Lung Electroporation Studies*  
Ian A. Pringle, Lee L. A. Davies, Gerry McLachlan, David D. Collie, Deborah R. Gill, Stephen C. Hyde

145

*Electroporation-Mediated IFN- $\beta$  Gene Transfer*  
Claire F. Evans, Maida M. de las Alas, Edward M. Nolan, Alain Luxembourg, Drew W. Hannaman, Gerald K. Nakamura, Barry L. Ellefsen, Robert M. Bernard

146

*Golden Cells: Gold Conjugate Labeling as a Potential Enhancer of Electro-Gene Transfer*  
Feng Liu, C. E. Polley, L. B. Stiger, S. Majetich, Leaf Huang

147

*Chemical Enhancers of Non-Viral Salivary Gland Gene Transfer*  
Beth M. McMahon, Yen-Ju Chen, Edmund J. Niedzinski, Michael J. Bennett

148

*The Altered Binding Properties of Sleeping Beauty Transposase Hyperactive Mutants May Explain Their Enhanced Efficacy*  
Julie Park, Stephen R. Yant, Mark A. Kay

149

*Anti-Idiotypic CDR3 Vaccination Against Chronic B-Cell Lymphoma: DNA Vaccine Strategy*  
Monica Rinaldi, Sandra Iurescia, Daniela Fioretti, Emanuela Signori, Simona Cali, Gisella Volpe, Claudia Casnici, Ornella Marelli, Giuseppe Saglio, Giancarlo Tonon, Vito Michele Fazio

150

*Cooperative Strand Invasion of Super-Coiled Plasmid DNA by Mixed Linear PNA and PNA-Peptide Chimeras*  
Karin E. Lundin, Rongbin Ge, Mathias G. Svahn, Elisabeth Tornquist, Lars J. Branden, C. I. Edvard Smith

151

*Gene Delivery in Larger Animals*  
Torunn Elisabeth Tjelle, Rune Kjeken, Ragnar Salte, Jacob Mathiesen

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Thursday, June 3

**152**

*Nucleofection and Phage phiC31 Integrase Mediate Stable Introduction of a Dystrophin Fusion Gene into Muscle Derived Stem Cell and Human Myoblasts*  
Simon P. Quenneville, Pierre Chapdelaine, Joel Rousseau, Nicolas J. Caron, Daniel Skuk, Eric C. Olivares, Michele P. Calos, Jacques P. Tremblay

**153**

*VEGF Associated with TP To Refine Angiogenesis in Gene Therapy*

Anna Rita Bellu, Marianne G. Rotz, Diane Bouïs, Nanno H. Mulder, Wendy Dam, Vincent van Weel, Pieter Koolwijk, Paul H. A. Quax, Hidde J. Haisma, Geke A. P. Hosper

**154**

*Small Inhibitory RNA Expressed from Sleeping Beauty Transposons Can Reduce alpha1-Antitrypsin Expression*  
Kirk J. Wangensteen, Zongyu J. Chen, Clifford J. Steer, Stephen C. Ekker

**155**

*Non-Viral Gene Therapy for Type 1 Diabetes by Electroporation of Insulin and Glucokinase Genes to Skeletal Muscle*

Joel Montane, Alexandre Mas, Miquel Chillon, Xavier M. Anguela, Sergio Muñoz, Efren Riu, Pedro J. Otaegui, Fatima Bosch

**156**

*Delivery of Plasmid DNA to the Nucleus Using Non-Viral Gene Transfection in Tumor Cell Lines and Primary Tumors*

Jill A. Gershman, James J. Weber, Rimas J. Orentas

**157**

*Clinical Evaluation of Pain and Muscle Damage Induced by Electroporation of Skeletal Muscle in Humans*

Rune Kjeken, Torunn Elisabeth Tjelle, Dag Kvale, Jacob Mathiesen

### Inborn Errors of Metabolism: Hemophilia and Other Diseases

**158**

*Transient Immunosuppression by CTLA4Ig and Anti-CD40L Promotes Long-Term Tolerance Specifically Against HFVIII in Hemophilia A Mice Following Naked DNA Transfer*

Peiqing Ye, Ling Zhou, Zhenping Shen, Arthur R. Thompson, David J. Rawlings, Hans D. Ochs, Carol H. Miao

**159**

*Vector Genome Loss Does Not Account for Decline of Plasma cFVIII Protein in Hemophilic Dogs That Demonstrate Long Term Clinical Correction Following Helper-Dependent Adenoviral Gene Therapy*  
W. Michael McCormack, Jr, Terry K. Bertin, Kiran Ubhayakar, Margaretha Guenther, Viraj P. Mane, Philip Ng, Timothy C. Nichols, Milton J. Finegold, Donna Palmer, Arthur Beaudet, Brendan H. Lee

**160**

*Overcoming the Size Limitations of Gene Transfer Vectors: Using Segmental Trans-Splicing To Deliver the Coding Sequence of Von Willebrand Factor*  
Robert G. Pergolizzi, Rachel Dragos, Katsutoshi Nakayama, Denisa Wagner, Ronald G. Crystal

**161**

*Novel Pseudotyped AAV Vectors for Hemophilia B Gene Therapy*  
Lili Wang, Jean-Pierre Louboutin, Timothy C. Nichols, James M. Wilson

**162**

*Neonatal Gene Therapy Induces Tolerance in Dogs but Not Cats*  
Lingfei Xu, Patty O'Donnell, Tom O'Malley, Mark E. Haskins, Wan-Ching Chan, Yuli Liu, Katherine P. Ponder

**163**

*Phenotypic Correction of a Hemophilia B Dog Following Systemic Administration of a Helper-Dependent Ad Vector Encoding Canine Coagulation Factor IX*  
Nicola Brunetti-Pierri, Donna Palmer, Timothy C. Nichols, Arthur L. Beaudet, Philip Ng

**164**

*Secretion of Human Recombinant Factor IX from Cells of Adipose, Myogenic and Hepatic Origin Transduced with Replication-Defective HSV-1 Genomic Vectors*  
Julie Fradette, Ryan Flanigan, David M. Krisky, William F. Goins, Joseph C. Glorioso

**165**

*Long-Term Factor VIII Expression and Phenotypic Correction in a Murine Model of Hemophilia A Achieved by Immunosuppression and Non-Viral Gene Transfer Using the Sleeping Beauty Transposon*  
John R. Ohlfest, Scott Perkinson, Paul Lobitz, Joel Frandsen, R. Scott McIvor, Perry Hackett, Karl Clark, Jeff Essner, Nigel Key, David A. Largaespada

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Thursday, June 3

**166**

*Induction of Immune Tolerance and Phenotypic Rescue of Hemophilic Mice by Stem Cell Mediated Gene Transfer*

Brian W. Bigger, Elena K. Siapati, Ajay Mistry, Simon N. Waddington, Megha S. Nivsarkar, Maxine V. Holder, Chris Ridler, Geoff Kemball-Cook, Robyn R. Ali, Stuart Forbes, Charles Coutelle, Nick Wright, Malcolm Alison, Adrian Thrasher, Dominique Bonnet, Michael Themis

**167**

*Partial Correction of the Hemophilic Phenotype in Mice Implanted with Encapsulated Human Primary Myoblasts Secreting FIX*

Jianping Wen, Frederick A. Ofosu, Gonzalo Hortelano

**168**

*EIAV-Based Vectors for the Treatment of Haemophilla A*

Pippa A. Radcliffe, Fraser J. Wilkes, Georgina L. Beard, Susan M. Kingsman, Kyriacos A. Mitrophanous

**169**

*Intrapancreatic Injections of Adeno-Associated Virus Vectors for Local Gene Expression*

Scott A. Loiler, Qiushi Tang, Marcel Perret-Gentil, Martha L. Campbell-Thompson, Mark A. Atkinson, Terence R. Flotte

**170**

*Ex Vivo Gene Therapy of Metabolic Liver Disorders Using Uncultured and Lentivirally Transduced Hepatocytes*

Tuan Huy Nguyen, Jacques Birraux, Barbara Wildhaber, Catherine Fux-Cametti, Claude Le Coultre, Didier Trono

**171**

*Enhancing Hepatocyte Gene Transfer with Baculovirus GP64 Glycoprotein*

Yubin Kang, Litao Xie, Diane Thi Tran, Melissa Hickey, Colleen S. Stein, Beverly L. Davidson, Paul B. McCray, Jr

**172**

*Comparison of Adenoviral and Adeno-Associated Viral Vectors for Pancreatic Gene Delivery In Vivo*

Alfred Y. Wang, Peter D. Peng, Anja Ehrhardt, Theresa A. Storm, Mark A. Kay

**173**

*Adenovirus-Mediated Gene Transfer to Mouse and Dog Pancreas In Vivo*

Eduard Ayuso, Miguel Chillon, Felix Garcia, Judith Agudo, Anna Andaluz, Virginia Haurigot, Assumpcio Bosch, Pedro J. Otaegui, Fatima Bosch

**174**

*High Efficiency HDAd-Mediated Hepatic Transduction Can Be Achieved by Delivery into the Surgically Isolated Liver of Nonhuman Primates*

Nicola Brunetti-Pierri, William Cioffi, David Iannitti, Thomas Ng, Donna Palmer, Arthur Beaudet, Milton Finegold, Karen Rice, Dee Carey, Philip Ng

**175**

*A Clinical Trial in Japan of Retroviral-Mediated Gene Transfer to Bone Marrow CD34+ Cells as a Treatment of Adenosine Deaminase (ADA)-Deficiency*

Makoto Otsu, Tadashi Ariga, Yoshihiro Maeyama, Jukei Yoshida, Satoru Nakajima, Miyuki Kida, Nariaki Toita, Norikazu Hatano, Nobuaki Kawamura, Motohiko Okano, Ryouji Kobayashi, Osamu Tatsuzawa, Masafumi Onodera, Fabio Candotti, Kunihiko Kobayashi, Yukio Sakiyama

**176**

*In Vivo Selective Enhancement of Correction of Human X-Linked Chronic Granulomatous Disease Neutrophils in NOD/SCID Mice Transplanted with CD34+ Stem Cells Transduced with a Bicistronic gp91phox/Benzyl Guanine Resistant Methylguanine Methyltransferase Vector*

Uimook Choi, Kouhei Yamashita, Lanise Caldwell, Harry L. Malech

**177**

*Lentiviral Vector Mediated ADA Gene Transfer in ADA-Deficient SCID Mice*

Denise A. Carbonaro, Xiangyang Jin, Dinithi Senadherra, Karen Pepper, Denise Petersen, Xiao-Jin Yu, Dianne Skelton, Donald B. Kohn

**178**

*Site-Specific Base Alteration in the Promoter Region of the Human Gamma Globin Gene by Single-Stranded Oligonucleotides for Gene Therapy of Sickle Cell Disease*

W. Yin, B. T. Kren, C. J. Steer

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

179

*Oncoretroviral Vector-Mediated Gene Therapy for Murine WAS Fails To Correct Most of the Deficient Biological Responses to S. pneumonia or M. bovis*  
Ted S. Strom, Jon McCullers, Robert Rutschman, Peter Murray, Marguerite Evans-Galea, John M. Cunningham, Arthur W. Nienhuis

180

*Use of Skeletal Muscle for Regulated Expression of Parathyroid Hormone To Treat Hypoparathyroidism*  
Maja Z. Salva, Jeffrey Bonadio, Stephen D. Hauschka

181

*Human CBS (Cystathionine  $\beta$ -Synthase) Gene Transfer Mediated by Recombinant Adeno-Associated Virus Vector*  
Hyun-Jeong Oh, Eun-Sook Park, Warren Kruger, Sung-Chul Jung, Jin-Sung Lee

182

*Towards the Correction of the Genetic Defect in Corneal Keratinocytes from Patients with Macular Corneal Dystrophy Type II*  
Stefano Ferrari, Giuliana Ferrari, Claudia Rossi, Annarita Miccio, Nicola Volpi, Fulvio Mavilio, Graziella Pellegrini, Michele De Luca

183

*Hemochromatosis: AAV Coding an Antisense Gene Against DMT-1 Iron Transporter in Intestinal Epithelial Cells Acts as RNAi. And Inhibits Iron Transport*  
Fernando E. Ezquer, Marco T. Nunez, Yedy Israel

184

*Evaluation of Cystine Transport in Cultured Human Kidney Cells: Primer to Gene Therapy of Cystinuria*  
Sreedhar Sagi, Thomas Knoll, Axel Schaaf, Peter Alken, Maurice Stephan Michel

185

*Systemic Protein Delivery by Muscle-Directed Gene Transfer Is Limited by a Local Immune Response*  
Lixin Wang, Eric Dobrzynski, Ou Cao, Roland Herzog

186

*Phenotypic Correction of Hemophilic Mice Using Hematopoietic Stem Cells Transduced with an MSCV-Based Oncoretroviral Vector*  
Morvarid Moayeri, Ali Ramezani, Richard A. Morgan, Teresa S. Hawley, Robert G. Hawley

Thursday, June 3

### Angiogenesis and Vascular Gene Therapy

187

*Adeno-Associated Virus Vector-Mediated Soluble Flt-1 Gene Transfer Prevents Diabetic Retinopathy in Spontaneously Diabetic Rat Model*  
Junichi Ideno, Hiroaki Mizukami, Akihiro Kakehashi, Yuka Saito, Takashi Okada, Akihiro Kume, Masatoshi Kuroki, Masanobu Kawakami, Shun Ishibashi, Keiya Ozawa

188

*Effects of Long-Term Expression of VEGF-A and Sema3A after Gene Delivery In Vivo Using AAV Vectors*  
Serena Zacchigna, Patarini Lucia, Arsic Nikola, Zentilin Lorena, Tomasi Sara, Dapas Marina, Giacca Mauro

189

*Sendai Virus Mediated Angiopoietin-1 Gene Therapy for Cardiovascular Diseases*  
Yoshinori Ito, Jinghua Huang, Takeshi Uzuka, Yukiko Honma, Hironari Dehari, Masayoshi Kobune, Kiminori Nakamura, Makoto Inoue, Mamoru Hasegawa, Hirofumi Hamada

190

*Myocardial Gene Therapy by Naked Angiopoietin-1 Plasmid Injection Efficiently Induces Angiogenesis and Prevents the Remodeling Following Acute Myocardial Infarction*  
Takeshi Uzuka, Yoshinori Ito, Masayuki Morikawa, Jinghua Huang, Masayoshi Kobune, Kiminori Nakamura, Yukiko Honma, Keiji Ishii, Tomio Abe, Hirofumi Hamada

191

*Improvement of Myocardial Dysfunction by Therapeutic Angiogenesis Induced by Transfection of HGF Gene Using Left Ventricular Electromechanical Mapping in Porcine Chronic Myocardial Infarction Model*  
Junya Azuma, Yosiaki Taniyama, Motokuni Aoki, Yasuhumi Kaneda, Toshio Ogihara, Ryuichi Morisita

192

*Cardiac-Specific and Hypoxia-Responsive Vascular Endothelial Growth Factor Expression in Ischemic Hearts*  
Hua Su, Yu Huang, Alicia Barcena, Janice Arakawa-Hoyt, Shuji Joho, Richard Sievers, William Grossman, Yuet Wai Kan

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Thursday, June 3

**193**

*Hypoxia-Inducible VEGF Gene Therapy Using the RTP801 Promoter*  
Donghoon Choi, Minhyung Lee, David A. Bull, Russel Reiss, Chien-Wen Chang, Lane Christensen, Sung Wan Kim

**194**

*Deltavasc™ - Phase I Study of Intramuscular Administration of Plasmid Del-1 (Developmentally Regulated Endothelial Cell Locus –1) in Humans with Peripheral Arterial Disease (PAD)*  
Sanjay Rajagopalan, Jeffrey Snell, Stuart W. Young, Gary L. Schaer

**195**

*Therapeutic Angiogenesis in Mouse Ischemic Heart after Intramyocardial Injection of a Novel Two Gene Plasmid Vector Co-Expressing High Levels of Human VEGF and bFGF Genes*  
Phoebe Luo, Kengo Kusano, Tom Tsang, David Harris, Douglas Losordo

**196**

*Regional Angiogenesis Mediated by Platelet-Derived VEGF in Response to Thrombopoietin Gene Transfer*  
Hideki Amano, Neil R. Hackett, Koji Shido, Rafael Tejada, Scott Avegilla, Shahin Rafii, Ronald G. Crystal

**197**

*Do Bone Marrow-Derived Progenitor Cells Contribute to VEGF-Induced Post-Natal Neovascularization?*  
Lorena Zentilin, Sabrina Tafuro, Nikola Arsic, Serena Zacchigna, Milena Sinigaglia, Sara Tomasi, Lucia Patarini, Marina Dapas, Mauro Giacca

**198**

*Cell-Based Gene Therapy for Primary Pulmonary Hypertension Using a Non-Viral Gene Delivery System*  
Linda N. Liu, Linhong Li, Yidan Zhao, Qiuwang Zhang, Rama Shivakumar, Cornell Allen, Joseph C. Fratantoni, Duncan J. Stewart, David W. Courtman

**199**

*Circulating Endothelial Progenitor Cells Restore Endothelial Function of Balloon Injured Rabbit Carotid Artery*  
Tongrong He, Leslie A. Smith, Noel M. Caplice, Zvonimir S. Katusic

**200**

*Cholesterol Modified Polyurethane as a Matrix for Cell Based Cardiovascular Therapy Using Blood Outgrowth Endothelial Cells (BOEC): Potential for Lentivirus Mediated Gene Therapy*  
Stanley J. Stachelek, Ivan S. Alferiev, Phillip W. Zoltick, Jeanne M. Connolly, Richard W. Bianco, Robert P. Hebbel, Robert J. Levy

**201**

*Angiogenic Gene Therapy of Coronary Artery Disease: From Bench to the Clinic*  
Maciej Malecki, Piotr Kolsut, Zbigniew Religa, Przemyslaw Janik

**202**

*Using RNA To Inhibit Intimal Hyperplasia by Blocking E2F Function*  
Alice K. Tanner, Joseph Nevins, Bruce Sullenger

**203**

*Electroporation-Mediated Gene Delivery to Mouse Femoral Arteries in a Model of Vascular Injury*  
Jennifer L. Young, Daniel Eefting, Paul H. A. Quax, David A. Dean

**204**

*Angiographic Luminal Loss in Porcine Coronary Arteries Is Reduced by Stent-Based Adenovirus-Mediated Antagonism of TGF- $\beta$ 1*  
Clare E. Appleby, Paul A. Kingston, Michel Verhoeven, Anita Driessen, Anthony M. Heagerty

**205**

*Histopathological and Physiological Impact on Monkey Intracranial Aneurysms Transfected Transforming Growth Factor-Beta-1 Mediated by Adenovirus*  
Shigeki Ono, Tomoyuki Ogawa, Tomohito Hishikawa, Tomotsugu Ichikawa, Isao Date

**206**

*Adenovirus-Mediated GTP Cyclohydrolase I Gene Delivery Augments Tetrahydrobiopterin and Restores Nitric Oxide Synthase Function in Hyperglycaemic Human Aortic Endothelial Cells*  
Shijie Cai, Keith M. Channon

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

#### Central Nervous System I

**207**

*Micro RNAs as Novel siRNA Shuttles*

Ryan Boudreau, Haibin Xia, Beverly L. Davidson

**208**

*Regenerative Properties of Retinoic Acid Receptor  $\beta 2$  in Rat Model of Dorsal Rhizotomy*

Liang-Fong Wong, Ping K. Yip, Anna Battaglia, John Grist, Lucy E. Walmsley, Malcolm Maden, Susan M. Kingsman, Alan J. Kingsman, Stephen B. McMahon, Nicholas D. Mazarakis

**209**

*Gene Silencing of Human Huntingtin Using Lentivirus-Delivered shRNA*

Scott Q. Harper, Patrick D. Staber, Christine R. Rowley, Xiaohua He, Ines H. Martins, Qinwen Mao, Henry L. Paulson, Beverly L. Davidson

**210**

*AAV Vector-Mediated Enzyme Replacement Therapy Is Efficacious in Reversing Storage Pathology and Preventing Purkinje Cell Death in a Mouse Model of Niemann-Pick A Disease*

Marco A. Passini, Jie Bu, James C. Dodge, Qi Zhao, Tatyana V. Taksir, Holly A. Collins, Catherine R. O'Riordan, Gregory R. Stewart

**211**

*Creation of a HSV VP22-Sleeping Beauty Fusion Protein To Facilitate Integration of HSV-1 Amplicon Vectors*

Hilary A. Stanley, William J. Bowers, Michael A. Mastrangelo, Howard J. Federoff

**212**

*AAV-Mediated Gene Transfer Protects Against Mitochondrial Optic Neuropathy in Mice*

Xiaoping Qi, Liang Sun, John Guy, William W. Hauswirth, Alfred S. Lewin

**213**

*Peripherally Delivered Glutamic Acid Decarboxylase (GAD) Gene Therapy for Below-Level Spinal Cord Injury Neuropathic Pain*

Darren Wolfe, Liu Jun, Hao Shuanglin, Huang Shuhua, Marina Mata, Joseph Glorioso, David Fink

**Thursday, June 3**

**214**

*Additive Effects of HSV Vectors Expressing Proenkephalin, Interleukin-4, and Tumor Necrosis Factor Alpha Soluble Receptor To Treat Bone Cancer Pain*

James R. Goss, Christine Fetterolf, William Goins, Darren Wolfe, Shaohua Huang, David Krisky, Joseph C. Glorioso, Marina Mata, David J. Fink

**215**

*Dimerizer-Regulated AADC Expression with Two AAV Vectors in 6-OHDA Lesioned Rats*

Laura M. Sanftner, Victor M. Rivera, Lan Feng, Lori Berk, John R. Forsayeth, Tim Clackson, Janet Cunningham

**216**

*Gene Therapy of Huntington Disease Using RNA Interference and Sleeping Beauty Transposon*

Zongyu J. Chen, Xiaoming Ma, Philip Wong, Mikel Roe, Chang-won Park, Betsy T. Kren, Walter C. Low, Clifford J. Steer

**217**

*Widespread Distribution of Beta-Glucuronidase in the Mouse Brain Is Achieved after a Single Inoculation with a Neuroattenuated HSV Vector: Distribution Differs with Inoculation Site*

Bradford K. Berges, John H. Wolfe, Nigel W. Fraser

**218**

*Nonhuman Primate Models for Retinal and Choroidal Neovascularization Using AAV2-Mediated Overexpression of Vascular Endothelial Growth Factor*

Corinna Lebherz, Albert M. Maguire, Alberto Auricchio, Waixing Tang, Tomas S. Aleman, Zhangyong Wei, Rebecca L. Grant, Artur V. Cideciyan, Samuel G. Jacobson, James M. Wilson, Jean Bennett

**219**

*Prolonged Bioactive Transgene Expression Driven by the HSV Latency Active Promoter 2 (LAP2) in the Peripheral Nervous System*

Munmun Chattopadhyay, Darren P. Wolfe, William C. Goins, Shaohua Huang, Joseph C. Glorioso, Marina Mata, David J. FINK

**220**

*Prolonged Biologically Active Transgene Expression Driven by HSV Latency Active Promoter 2 (LAP2) in Brain In Vivo*

Veljko Puskovic, Darren Wolfe, James R. Goss, Shaohua Hang, Joseph C. Glorioso, Marina Mata, David J. Finn

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Thursday, June 3

**221**

*Developing HSV-Mediated Gene Therapy Vectors To Adjust the Metabolic Imbalance of A $\beta$  in Alzheimer's Disease*

Chang-Sook Hong, James R. Goss, William F. Goins, David J. Fink, Joseph C. Glorioso

**222**

*HSV Amplicon Expression of Single Chain Antibodies Directed Against  $\alpha$ -Synuclein Conformers*

Kathleen A. Maguire-Zeiss, Eric Yehling, Rita Giuliano, Mark Sullivan, Howard J. Federoff

**223**

*Delivery of HSV Based Vectors for Correction of CNS Pathology in Mucopolysaccharodosis Type vii (MPS vii)*

Srikanth Yellayi, Bradford Berges, John Wolfe, Charles Vite, Nigel Fraser

**224**

*Adenoviral Co-Delivery of BDNF and Noggin Induces Striatal Neuronal Replacement and Delays Motor Impairment in a Transgenic Model of Huntington's Disease*

Sung-Rae Cho, Eva Chmielnicki, Steven A. Goldman

**225**

*Phenotypic Correction of ALD Mouse after Hematopoietic Cell Transplantation and Evaluation of Hematopoietic Stem Cell Gene Therapy with a Lentivirus Vector*

Sonia Benhamida, Fran $\phi$ oise Fouquet, Ann Moser, Patrick Aubourg, Nathalie Cartier

**226**

*Developing a CNS-Specific Inflammatory Model To Investigate Gene Therapy/Inflammation Interactions in the Context of Neurodegeneration*

Michelle C. Janelsins, Michael A. Mastrangelo, Howard J. Federoff, William J. Bowers

**227**

*Anatomical and Behavioral Effects of AAV2-Mediated Delivery of Neurturin to the Rat Striatum Prior to 6-Hydroxydopamine Induced Nigrostriatal Degeneration*

Christopher D. Herzog, Mehdi Gasmi, Justine J. Cunningham, Eugene P. Brandon, Anthony Ramirez, Alistair Wilson, Eva K. Hofer, Jennifer Hwang, Leo Manlapaz, Jeffrey H. Kordower, Jeffrey M. Ostrove, Raymond T. Bartus

**228**

*A Novel Therapeutic Strategy To Treat Brain Ischemia: Over-Expression of Hepatocyte Growth Factor Gene Reduced Ischemic Injury without Cerebral Edema in Rat Model*

Munehisa Shimamura, Naoyuki Sato, Toshio Ogihara, Yasufumi Kaneda, Ryuichi Morishita

**229**

*AAV4 Mediated Gene Transfer in Mucopolysaccharidosis Type VII Mice*

Gumei Liu, John A. Chiorini, Beverly L. Davidson

**230**

*Neural Progenitor Cell Transduction with AAV Serotypes 1 and 4*

Gumei Liu, Robert M. Kotin, John A. Chiorini, Beverly L. Davidson

**231**

*Supplementation of Peripherin/rds Rescues Mutation-Associated Rod and Cone Photoreceptor Defects in Transgenic Mice*

May Nour, Muna I. Naash, Steven J. Fliesler

**232**

*Transplantation of Human Embryonic Stem Cell Derived Oligodendroglial Progenitors for the Treatment of Spinal Cord Injury*

Jane Lebkowski, Gabriel Nistor, Giovanna Bernal, Minodora Totoiu, Catherine Priest, Scott Thies, Jerrod Denham, Hans Keirstead

**233**

*Gene Control as a Therapeutic Intervention: Zinc-Finger Protein Transcription Factors as Regulators of the Molecular Determinants of Neuropathic Pain*

Yann Jouvenot, John R. Forsayeth, Siyuan Tan, Andrew McNamara, Raymond A. Chavez, Philip Gregory

**234**

*Correction of Pathology in Galactosialidosis Mice Crossed into Transgenic Mice Expressing PPCA in Neural Cells*

Huimin Hu, Linda Mann, Erik Bonton, Taylor Walker, Alessandrada d'Azzo

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

#### Muscle

**235**

*The Fetal Approach: A Novel Therapy for the Treatment of Musculo-Skeletal Disease*  
Michael Themis, Lisa G. Gregory, Simon N. Waddington, Maxine V. Holder, Kyriacos A. Mitrophanous, Suzanne M. K. Buckley, Brian W. Bigger, Fiona E. Ellard, Lucy E. Walmsley, Pippa Radcliffe, Nick Mazarakis, Mimoun Azzouz, Lorraine Lawrence, Terrence Cook, Faisal A. Allaf, Susan Kingsman, Charles Coutelle

**236**

*Delivery of VEGF Using AAV Vectors Stimulates Skeletal Muscle Regeneration In Vivo*  
Nikola Arsic, Serena Zacchigna, Lorena Zentilin, Genaro Ramirez-Correa, Alessandro Salvi, Gianfranco Sinagra, Mauro Giacca

**237**

*Transgenic Expression of Dp116 in Muscle Does Not Ameliorate Dystrophy in mdx4cv Mice*  
Luke M. Judge, Jeffrey S. Chamberlain

**238**

*A Comparison of Gene Delivery and Vector Systems To Achieve High Levels of Sustained Skeletal Muscle Gene Expression*  
Hu Sheng Qian, Perry Liu, Paul Szymanski, Peter Kretschmer, Alan Brooks, Maxine Bauzon, Fang Jin, Richard N. Harkins, Richard J. Samulski, Gabor M. Rubanyi, Terry W. Hermiston

**239**

*Human Myoblast Deimmortalization Using Tat-Mediated Cre Recombinase Delivery*  
Nicolas J. Caron, Marie-Eve Ducharme, Philippe Mills, Simon P. Quenneville, Jacques P. Tremblay

**240**

*Immunogenicity of Dystrophins Delivered to Mice by Gutted Adenoviral Vectors*  
Jie Mi, Leonard Leuse, Jeannine Scott, Shannon Barker, Shanna Sawatzki, Dennis Hartigan-O'Connor, Jeffrey S. Chamberlain

**241**

*Lentivirus Mediated Dystrophin Expression in mdx Muscles*  
En Kimura, Sheng Li, Brent Fall, Sanna Sawatzki, Leonard Meuse, Jeffrey S. Chamberlain

**Thursday, June 3**

**242**

*A Novel MiniDys-eGFP Fusion Gene for Developing Cell-Based Therapies of Duchenne Muscular Dystrophy*  
Sheng Li, En Kimura, Leonard Meuse, Xin Ye, Brent Fall, Steven D. Hauschka, John Faulkner, Jeffrey S. Chamberlain

**243**

*Delivery of Igf-1 and Dystrophin to Dystrophic mdx Muscle*  
Simone Abmayr, Paul Gregorevic, James M. Allen, Shanna M. Sawatzki, Jeffrey S. Chamberlain

**244**

*Successful AAV Vector-Mediated Gene Transfer into Canine Skeletal Muscle Required Suppression of Excess Immune Responses*  
Katsutoshi Yuasa, Madoka Yoshimura, Nobuyuki Urasawa, Katsujiro Sato, Yuko Miyagoe-Suzuki, John McC Howell, Shin'ichi Takeda

**245**

*Muscle-Derived Stem Cells Display an Extended, but Not Unlimited, Expansion Capability: Implication for Muscle Regeneration*  
Bridget M. Deasy, Burhan M. Gharaibeh, Michele Jones, Michael A. Lucas, Johnny Huard

**246**

*Gender Differences in Transplantation Efficiency Using Muscle-Derived Stem Cells for Muscular Dystrophy*  
Bridget M. Deasy, Aiping Lu, Burhan M. Gharaibeh, Michele Jones, Jessica Tebbets, Johnny Huard

**247**

*Expressing Full-Length Dystrophin in 50% Cardiomyocytes Corrects Cardiomyopathy in the Mdx Mouse Model for Duchenne Muscular Dystrophy*  
Yongping Yue, Jeffrey W. Skimming, Mingju Liu, Yujiang Fang, Tammy Strawn, Dongsheng Duan

**248**

*cFLIP or IKBSR Inhibition of NF- $\kappa$ B Activation in Mouse Myoblast Cells: Potential Application of Gene Therapy for Cancer Cachexia*  
Zhilong Jiang, Paula R. Clemens

**249**

*New Canine Models of Duchenne Muscular Dystrophy: Identification and Molecular Characterization*  
Bruce F. Smith, Lucia Alvarez, Kerriann Sparks

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Thursday, June 3

**250**

*Cross-Correction in Pompe Mice after Immunomodulation and Hepatic Delivery of Recombinant AAA5 and AAV8 Vectors*  
Kerry O. Cresawn, Clive Wasserfall, Mark A. Atkinson, Cathryn Mah, Thomas J. Fraites, Jr, Irene Zolotukhin, Barry J. Byrne

**251**

*rAAV-Mediated Gene Therapy To Treat Limb Girdle Muscular Dystrophy Type 2D (LGMD-2D)*  
Christina A. Pacak, Denise Cloutier, Irene Zolotukhin, Gabriel S. Gaidosh, Kevin Campbell, Glenn A. Walter, Barry J. Byrne

### Cancer Immunotherapy

**252**

*Phase II Study of MVA Expressing E6 and E7 of HPV16 in Patients with CIN2/3*  
Patrick Squiban, Patrice Mathevet, Bernard Huynh, Jean-Pai; Bory, Jean-Marc Balloul

**253**

*Regression of Large Solid Tumors Using Engineered Drug Resistant Immunocompetent Cells and Selective Chemotherapy*  
Douglas W. McMillin, Becker Hewes, Bagirath Gangadharan, H. Trent Spencer

**254**

*Vaccination of Recurrent Glioma Patients with Tumor Lysate-Pulsed Dendritic Cells Elicits Immune Responses: Results of a Clinical Phase I/II Trial*  
Ryuya Yamanaka, Junpei Homma, Naoki Yajima, Naoto Tsuchiya, Ryuichi Tanaka

**255**

*Potent Cancer Immunotherapy with Dendritic Cells Generated from Genetically Modified Hematopoietic Stem Cells*  
Tsukasa Nabekura, Hiromitsu Nakauchi, Toshiro Nagasawa, Masafumi Onodera

**256**

*Importance of Temporal Control of HSVtk Gene Therapy in Combination with T Cell Based Therapies for Cancer*  
Luis A. Sanchez-Perez, Michael Gough, Jill Thompson, Richard G. Vile

**257**

*AAV Vector-Loading DC with an Autoimmune Domain-Depleted HCV Core Gene Stimulates High Anti-Core and Low Anti-Self CTL*  
Maurizio Chiriva-Internati, Yong Liu, Rongcheng Luo, Changxuan You, Paul L. Hermonar

**258**

*Multi-Modality Non-Invasive Imaging of NK-92 Cells Trafficking and Tumor Targeting in Mice*  
Vilia Tourkova, Michael Doubrovic, Malgorzata Dabrowska, Jelena Vider, Shangive Cai, Ronald Finn, Juri Gelovani Tjuvajev, Vladimir Ponomarev

**259**

*In Vivo Suicide Gene Therapy of Human T Lymphocytes in a Murine Xenograft Model*  
Bruno Nervi, Michael Rettig, Julie Ritchey, John Walker, Gerhard Bauer, Philip Herrbrich, Jan A. Nolta, John F. DiPersio

**260**

*Anti-Tumor Response by Adenovirus-Mediated, Tumor Specific Expression of a Pro-Apoptotic Protein (TRAIL) and a Heat Shock Protein (GRP94)*  
Nelson C. Di Paolo, Shaoheng Ni, Andre Lieber

**261**

*Immune Evasion in Gene Therapy*  
Rob C. Hoeben, Martine Ossevoort, Birgit M. Visser, Arnaud Zaldumbide, Ellen I. Van der Voort, Diana J. Van den Wollenberg, Rene E. Toes

**262**

*Dendritic Cell (DC) Vaccine Loaded with Apoptotic Tumor Fragments Effectively Suppress Preestablished Malignant Melanoma in Mice*  
Hidetsugu Asada, Tsunao Kishida, Hideyo Hirai, Jiro Imanishi, Osamu Mazda

**263**

*Antigen Epitope-Expressing Cytokines for DNA Immunization*  
Xianghui He, Tom C. Tsang, Tong Zhang, Phoebe Luo, David T. Harris

**264**

*Non-Invasive Monitoring of the Response of Orthotopic Bladder Tumors to GM-CSF Gene Therapy Using the Prostate Specific Antigen Gene as a Reporter*  
Qinghui Wu, Ratha Mahendran, Kesavan Esuvaranathan

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

**265**

*ImmunoVexEx Vivo: A Herpes Simplex Virus-Based, Dendritic Cell-Mediated Anti-Cancer Vaccine Platform*

Philip Reay, Mei Yi-Sun, Binlei Liu, Yvonne McGrath, Madeleine Wakeling, Justin John, Emma Turnbull, Suzanne Thomas, Rob Coffin

**266**

*OncovEX: A Family of Oncolytic Herpes Simplex Viruses Optimised for Therapeutic Use*

Binlei Liu, Ziquin Han, Guy Simpson, Rob Coffin

**267**

*Enhancement of T-Cell Immunity by CEA Delivery to Dendritic Cells with Adenovirus Retargeted Via sCAR-Fibritin-CD40L*

Papia T. Banerjee, Stephan G. Klinz, Alexander V. Pereboev, Douglas R. Nelson, James S. Huston, David T. Curiel, Stephen D. Gillies

**268**

*Enhancement of DNA Vaccine Potency by Coadministration of a Tumor Antigen Gene and DNA Encoding Serine Protease Inhibitor-6*

Chien-fu Hung, Taewoo Kim, T. C. Wu

**269**

*Targeting Self Antigens through Allogeneic TCR Gene Transfer*

Moniek de Witte, Miriam Cocciris, Monika C. Wolkers, Marly van den Boom, Helmut W. Kessels, Ton N. Schumacher

**270**

*Treatment of CT26 Tumors with Dendritic Cells Transduced with a Recombinant SV40 Vector Expressing Interleukine 15*

Maria Vera, Gloria G. Aseguinolaza, Nerea Razquin, Ignacio Melero, Jesus Prieto, Puri Fortes

**271**

*Helper-Dependent, Capsid-Modified Adenoviral Vectors for Immunotherapy of Breast Cancer*

Nelson C. Di Paolo, Daniel Stone, Dmitry Shayakhmetov, Andre Lieber

**272**

*Development of "Full"-Closed Sterile and Automatic Retroviral Gene Transfer System for Clinical Gene Therapy*

Shin Kaneko, Makoto Otsu, Yuichi Hasegawa, Hiroshi Kojima, Takashi Shimizu, Takashi Fukushima, Ryo Sumazaki, Masafumi Onodera, Toshiro Nagasawa

**Thursday, June 3**

**273**

*Long-Term Effects of Plasmid-Mediated Growth Hormone Releasing Hormone in Dogs*

Catherine M. Tone, Dawn M. Cardoza, Robert H. Carpenter, Ruxandra Draghia-Akli

**274**

*Combination of a Second Generation Genetically Modified Herpes Simplex Virus 1 (HSV 1) with Paclitaxel in the Treatment of Breast Cancer In Vitro*

Jennifer Hu, G. Hallden, Claire Shorrock, Guy Simpson, Rob Coffin, T. Kamalati, R. Coombes

### Targeted Cancer Therapies I

**275**

*Prostate Targeting Peptides Via the Prostate Specific Membrane Antigen*

Shawn E. Lupold, Ronald Rodriguez

**276**

*Evaluation of an Oncolytic Adenovirus: Efficacy Studies on Primary Human Tumor Cell Cultures and Safety Studies in Non-Human Primates*

Shaoheng Ni, Anuj Gaggar, Pavel Sova, Robert Strauss, Nelson C. DiPaolo, Andre Lieber

**277**

*Variability in Infectivity of Primary Cell Cultures of Human Brain Tumors with HSV-1 Amplicon Vectors*

Maria A. Rueger, Alexandra Winkeler, Hrvoje Miletic, Christine Kaestle, Raphaela Richter, Gabriele Schneider, Juergen A. Hampl, Cornel Fraefel, Andreas H. Jacobs

**278**

*Hypoxia Reduces Adenoviral Replication in Cancer Cells by Down-Regulation of Viral Protein Expression*

Teona Pipiya, Harald Sauthoff, Yao Qi Huang, Benjamin Chang, William N. Rom, John G. Hay

**279**

*Methotrexate Chemotherapy of L1210 Leukemia in Mice Transplanted with Transgenic Marrow Expressing Drug-Resistant Dihydrofolate Reductase*

Joel L. Frandsen, Debra Swanson, R. Scott McIvor

**280**

*The Use of the Regulatory Sequences of H19 and IGF2 Genes in DNA Based Therapy of Colorectal Rat Liver Metastases*

Patricia Ohana, Pinhas Schacter, Basem Ayesh, Aya Mizrahi, Suhail Ayesh, Tatiana Birman, Tamar Schneider, Imad Maatouk, Peter J. Kuppen, Nathan de Groot, Abraham Czerniak, Abraham Hochberg

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Thursday, June 3

**281**

*Modulating the Immune Response to Oncolytic Measles Viruses*

Rae M. Myers, Mary E. Harvey, Diane C. Soeffker, Suzanne M. Greiner, Kah-Whye Peng, Stephen J. Russell

**282**

*Transient Adenoviral Methylpurine DNA Glycosylase Overexpression Imparts Chemotherapeutic Sensitivity to Human Breast Cancer Cells*

Mikael Rinne, David Caldwell, Ying He, Mark R. Kelley

**283**

*Adeno-Associated Viral Vector Expression Angiostatin and IL-15 for Brain Cancer Gene Therapy*

Horng Jyh Harn, Kuang Wen Liao, Shinn Zong Lin

**284**

*Oncolytic Adenovirus E1B-19kD Deletion and E3B Retention Results in Both Enhanced Potency and Tumor Necrosis Factor - Mediated Selectivity*

Ta-Chiang Liu, Yaohe Wang, Gunnel Hallden, Jennelle Francis, Gabriel Brooks, Nick Lemoine, David Kirn

**285**

*Evidence of Antiangiogenic and Antimetastatic Activities of the Recombinant Disintegrin Domain of Metargidin*

Veronique Trochon-Joseph, Dominique Renoir, Lluis M. Mir, Annick Thomaidis, Paule Opolon, Elisabeth Connault, Hong Li, Christophe Grenet, Francoise Fauvel-Lafeve, Jeannette Soria, Chantal Legrand, Claudine Soria, Michel Perricaudet, He Lu

**286**

*Conditionally Replicating Adenoviral Vectors for Glioblastoma Treatment*

Bertie H. C. G. M. de Leeuw, Maarten ter Horst, Mark Rodijk, Michael Brenner, Rob C. Hoeben, Peter A. E. Silleveld Smitt

**287**

*Enhancement of Therapeutic Efficacy of G207 for the Treatment of Malignant Glioma through Musashi1 Promotor Retargeting of ICP 34.5 Gene-Mediated Virulence*

Takahito Yazaki, Ryuichi Kanai, Hideyuki Tomita, Atsuo Shinoda, Hideyuki Okano, Takeshi Kawase

**288**

*Synergism Between a Conditionally Replicating Adenovirus Expressing p53 and Oxaliplatin in the Antiproliferative Effects on Human Colon Cancer Cell Lines*

Dinja Oosterhoff, Victor W. van Beusechem, Epie Boven, Herbert M. Pinedo, Winald R. Gerritsen

**289**

*Cisplatin and Doxorubicin Enhance the Activity of the CRAd Ad5-Δ24RGD Against Osteosarcoma Cell Lines but Not Primary Osteosarcoma Cells*

Harm C. A. Graat, M. Adhiambo Witlox, Frederik H. E. Schagen, Gertjan J. L. Kaspers, David T. Curiel, Marco N. Helder, Johannes Bras, Gerard R. Schaap, Winald R. Gerritsen, Paul I. J. M. Wuisman, Victor W. van Beusechem

**290**

*Demonstration of Antitumor Efficacy with an Oncolytic Adenovirus CG8840 for the Treatment of Bladder Cancer in an Orthotopic Tumor Model in Mice*

Nagarajan Ramesh, Ying Ge, Jun Liu, Deborah Farson, Melinda Van Roey, Virginia Rojas, Peter Working, DeChao Yu

**291**

*Enhanced Therapeutic Effect of Adenoviral Vector-Mediated RTVP-1 Gene Therapy in Combination with Irradiation for Prostate Cancer*

Tetsuo Fujita, Koji Naruishi, Nobuyuki Kusaka, Takefumi Satoh, Xiaorong Ji, Jianxiang Wang, Xiaohong Zhu, Guang Yang, Alexei A. Goltsov, Terry L. Timme, Maria T. Vlachaki, Bin S. Teh, E. Brian Butler, Timothy C. Thompson

**292**

*Modification of the p53 Transgene of a Replication-Competent Adenovirus Improves p53 Stability and Cancer Cell Killing*

Harald Sauthoff, Teona Pipiya, Shu Chen, Sheila Heitner, Yao Qi Huang, William N. Rom, John G. Hay

**293**

*The Epithelial Glycoprotein-2 Promoter Controls Selective and Efficient Killing of Epithelium Derived Cancer Cells*

Willemijn M. Gommans, Marianne G. Rots, Pamela M. J. McLaughlin, Simone J. van Eert, Martin C. Harmsen, Masato Yamamoto, David T. Curiel, Hidde J. Haisma

# The American Society of Gene Therapy's 7th Annual Meeting final program

2004

## Abstract Directory

**294**

*Herpes Simplex Virus Mediated Oncolysis Is an Effective Intratumoral Therapy in High-Risk Neuroblastoma Tumor Models*

Nehal S. Parikh, Mark A. Currier, Lisa C. Adams, Margaret H. Collins, Timothy P. Cripe

**295**

*Selective Breast Tumor Growth Suppression Mediated by the Enhanced Breast Cancer-Specific Promoter Chi-Ping Day, Kun-Ming Rau, Mien-Chie Hung*

**296**

*Combination Therapy with Adenovirus (Ad5) and Cytotoxic Drugs Can Act Synergistically To Induce Cell Death in Carcinoma Cell Lines in Culture*

Gunnel K. Hallden, Jiang Hui Meng, Richard Hill, Yaohe Wang, Jennelle Francis, Nicholas N. Lemoine, David H. Kirn

## Gene Regulation: Methods

**297**

*Development of HVJ-E High Throughput Gene Function Screening System: Screening of Endothelial Cell Growth Factors*

Tomoyuki Nishikawa, Hironobu Nakagami, Chiah Yian Yo, Yasufumi Kaneda

**298**

*RNA Mediated Transcriptional Gene Silencing in Human Cells*

Kevin V. Morris, Chris Chung, David J. Looney

**299**

*Insertion Site Analysis and In Vivo Selection*

Michael A. Harkey, Michael Weinreich, Yasuo Nagasawa, Patrick A. Navas, C. Anthony Blau

**300**

*Targeted Gene Correction in Mouse Embryonic Stem Cells Using Helper-Dependent Adenoviral Vectors*

Fumi Ohbayashi, Michael A. Balamotis, Ko Mitani

**301**

*CID-Mediated In Vivo Selection: Heterogeneity among Large Animal Models*

Robert E. Richard, R. Angelo De Claro, Sylvia Chien, Hans-Peter Kiem, Tim Clarkson, Robert Andrews, C. Anthony Blau

**302**

*Engineered Zinc Finger Transcription Factors: A Platform for Therapeutic Gene Regulation with Exquisite Specificity*

Dmitry Guschin, Steve Zhang, Lei Zhang, Siyuan Tan, Victor Bartsevich, Casey Case, Edward Rebar, Philip Gregory

**303**

*Impaired Nuclear Transport and Virus Uncoating Limit Recombinant Adeno-Associated Virus 2 Vector-Mediated Transduction of Primary Murine Hematopoietic Cells*

Li Zhong, Weiming Li, Zuocheng Yang, Njeri Maina, Keyun Qing, Yanjun Li, Linyuann Chen, Mengqun Tan, Rebecca J. Chan, Weihong Zhao, Steven H. Larsen, Mervin C. Yoder, Weinian Shou, Arun Srivastava

**304**

*Atelocollagen-Based Cell Transfection Array Allows High-Throughput Screening of Gene Functions*

Kimi Honma, Hiroshi Ito, Syunji Nagahara, Teruo Miyata, Takahiro Ochiya

**305**

*Regulation of Targeted Gene Repair by DNA Replication and by DNA Replication Arrest*

Eric B. Kmiec, Jennifer Sonntag, Erin Brachman

**306**

*Noninvasive Monitoring of Transgene Expression Using Surrogate Marker Peptides*

Linh D. Pham, Suzanne Greiner, Takafumi Nakamura, Kah-Whye Peng, Gabriela A. Rosales, Kent R. Bailey, Pai C. Kao, Stephen J. Russell

**307**

*A Mechanism for AZT Mediated Enhancement of Transgene Expression*

Fayaz R. Khazi, Andrew C. Edmondson, Katherine A. High

**308**

*A New Model to Functionally Measure Fusion Events in the Liver after Bone-Marrow Transplantation over Time*

Konrad L. Streetz, Denison D. Jenkins, Michael T. Longaker, Karl G. Sylvester, Mark A. Kay

**309**

*Controlling Embryogenesis Using Engineered Zinc Fingers in Drosophila*

Andrew C. Jamieson, Thomas J. Cradick, Bo Guan, Hong Xiao, Pamela M. Carroll

Thursday, June 3

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Thursday, June 3

**310**

*Feasibility and Limitations of Target Gene Repair in Mammalian Cells*  
Eric B. Kmiec, Li Liu, Hetal Parekh-Olmedo

**311**

*Targeted Gene Delivery to Selected Liver Segments by Isolated Hepatic Perfusion with Clamping of the Portal Vein*  
Hiroyasu Kinoshita, Sanae Hisayasu, Atsushi Watanabe, Satoru Suzuki, Takashi Shimada

**312**

*Developing Qualified Activity and Potency Assays for Gene Therapy and DNA Vaccine Products*  
Rick Hancock, Christopher Duffy

**313**

*Ligand-Specific Multiplex Gene Switch Expression in a Mouse Model*  
David W. Potter, Mohan B. Kumar, Martha A. DiPietro, Subba R. Palli

**314**

*mRNA Production Is the Major Factor Limiting Autogene-Based Cytoplasmic Expression*  
Jonathan D. Finn, Ian MacLachlan, Pieter R. Cullis

**315**

*Targeted Gene Repair Exhibits a Polar Bias in the Directionality of Correction*  
Eric B. Kmiec, Miya Drury

**316**

*Cystic Fibrosis Airway Epithelial Cells Transfection*  
Rosalie Maurisse, Yann Fichou, Judy Chen, Claude Ferec, Jian-Sheng Sun, Dieter C. Gruenert

**317**

*DNA Damage and Homologous Recombination Regulate the Frequency and Process of Targeted Gene Repair*  
Eric B. Kmiec, Luciana Ferrara, Katie Maguire, Hetal Parekh-Olmedo, Tim Schwartz

**318**

*Cationic Lipids/Ligands Induce DNA Helix Denaturation and Alter Transcriptional Activity*  
Prasad K. T., Madhusudhana N. Rao, Vijaya Gopal

**319**

*Improvement of Transgene Expression through the Direct Intrahepatic Injection of Adenoviral Vectors*  
Julien Swen Crettaz, Pedro Berraondo, Gloria Gonzalez-Aseguinolaza, Veijay Shankar, Laura Ochoa

**320**

*Effect of Sexual Steroid Hormones on Human egr-1 Promoter*  
Francisco Martinez-F., Hilda Villegas-C., Alejandro Jimenez-O., Roberto Razo, David T. Curiel

**321**

*siFECT™: Optimized Formulation for the Delivery of siRNA at Minimum Doses and Maximum Specificity*  
Sebastien Spagnou, Michael Jorgensen, Andrew D. Miller, Michael Keller

**322**

*Characterization of IFNy Gene Expressions in Rat Tissues with Real Time Quantitative RT-PCR*  
Baohong Tian, Sarah S. Rentz, Gregory S. Gorman, Rogers Tina, John G. Page

**323**

*Adenovirus Mediated Transfer of LacZ Gene in the Murine Melanoma Cell B16*  
Hui Li, Jiansheng Li

**324**

*The Degron Motif: A Protein Destruction Module with Potent Transcriptional Activation Properties for ZFP-Targeted Gene Regulation*  
Siyuan Tan, Dmitry Guschin, Carolyn Dent, Andreas Reik, Pei-Qi Liu, Philip D. Gregory

**325**

*Identification of Mechanistically Distinct Repression Domains at Endogenous Genes Using Engineered Zinc Finger Proteins*  
Siyuan Tan, Lei Zhang, H. Steven Zhang, Xaohong Zhong, Yann Jouvenot, Andrew W. Snowden, Andrew R. McNamara, Casey C. Case, Carl O. Pabo, Philip D. Gregory

**326**

*Zinc Finger Protein Targeted Epigenetic Gene Regulation: Toward Directed Long-Term Gene Control*  
Andrew R. McNamara, Andrew W. Snowden, Philip D. Gregory

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

#### RNA Virus Vectors: New Technologies

Room 205 A-D

**327 1:45 PM**

*Retargetable Oncolytic Measles Viruses for Cancer Virotherapy*

Takafumi Nakamura, Kah-Whye Peng, Mary Harvey, Stephen J. Russell

**328 2:00 PM**

*Paramyxovirus Fusion Protein Trimers: Partial*

*Membrane-Proximal Ectodomain Cleavage*

*Enhances Function*

Veronika von Messling, Dragana Milosevic, Patricia Devaux, Roberto Cattaneo

**329 2:15 PM**

*Long-Term Transgene Expression by Hepatocytes Transduced with Retroviral Vectors Requires Induction of Immune Tolerance to the Transgene*

Juliana Pupi, Carole Guillonneau, Virginie Picard, Maria C. Cuturi, Nicolas Ferry, Ignacio Anegon

**330 2:30 PM**

*Genetic Modification of Murine Embryonic Stem Cells by the Gene Silencing Resistance Retroviral Vector GCDsap*

Sanae Hamanaka, Joichi Usui, Satoru Takahashi, Hisahiro Yoshida, Michio Nagata, Makoto Otsu, Shin Kaneko, Toshiro Nagasawa, Hiromitsu Nakauchi, Masafumi Onodera

**331 2:45 PM**

*Human T Cells Efficiently Abort RCR-Infection through a CEM15/APOBEC3G-Mediated Mechanism*

Saskia B. Ebeling, Elles Simonetti, Stephanie Laufs,

Frank Giordano, Stefan Fruehauf, Anton Hagenbeek

**332 3:00 PM**

*Dual Gene Transfer and Transgenesis by Novel Lentiviral Vectors Carrying Synthetic Bi-Directional Promoters*

Mario Amendola, Mary Anna Venneri, Elisa Vigna, Luigi Naldini

**333 3:15 PM**

*Vector Development by Molecular Evolution: A Selection Procedure for Envelope Proteins*

*Pseudotyping Lentiviral Particles*

Christoph A. Merten, Joern Stitz, Klaus Cichutek, Christian J. Buchholz

**Friday, June 4**

**334 3:30 PM**

*Micro-Environmental Effects on Gene Transfer by Pseudotyped Lentiviral Vectors*

James R. Gilbert, Frank Park

**335 3:45 PM**

*Long-Term Genetic Modification of the Ocular Outflow Tract Coupled with Non-Invasive Real-Time Imaging of Gene Expression*

Nils A. Loewen, Michael P. Fautsch, Wu-Lin Teo, Cindy K. Bahler, Douglas H. Johnson, Eric M. Poeschla

**336 4:00 PM**

*Foamy Virus - Adenovirus Hybrid Vectors*

Axel Rethwilm, Marcus Picard-Maureau, Florian Kreppel, Dirk Lindemann, Thomas Juretzek, Ottmar Herchenroeder, Martin Heinkelein, Stefan Kochanek

#### AAV Vectors: Serotypes and Applications

Room 211 A-D

**337 1:45 PM**

*Unrestricted Liver Transduction with AAV Serotype 8 Vector in Mice*

Hiroyuki Nakai, Theresa Storm, Sally Fuess, Mark A. Kay

**338 2:00 PM**

*Therapeutic Levels for  $\alpha$ 1-Antitrypsin Following Intrapleural Administration of a Non-Human Primate Serotype rh10 AAV Vector Expressing  $\alpha$ 1-Antitrypsin*

Bishnu P. De, Nabil Wasif, Neil R. Hackett, Guangping Gao, James M. Wilson, Ronald G. Crystal

**339 2:15 PM**

*Sustained, High-Level Expression of Human Acid Alpha-Glucosidase and Correction of Glycogen Storage Disease Type II (GSD II) with an Adeno-Associated Virus 8 (AAV2/8) Vector Containing a Liver-Specific Promoter*

Luis M. Franco, Baodong Sun, Andrew Bird, Haoyue Zhang, Andrea Amalfitano, Y.-T. Chen, Dwight D. Koeberl

**340 2:30 PM**

*Assessment of the Transduction Patterns of Alternative AAV Vector Serotypes in the CNS of Rhesus Macaques*

Bruce A. Bunnell, Jason DuFour, Gretchen Belenchia, Xavier Alvarez, Kate Baker, Pete Didier, Andrew Lackner, John H. Wolfe

**341 2:45 PM**

*Autoimmune Anemia in Macaques Following Erythropoietin Gene Therapy*

Guangping Gao, Corinna Lebherz, Daniel J. Weiner, Rebecca L. Grant, Roberto Calcedo, Adam Bagg, Yi Zhang, James M. Wilson

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Friday, June 4

**342 3:00 PM**

*Self-Complementary Adeno-Associated Virus 2 (AAV)-T Cell Protein Tyrosine Phosphatase Vector as a Helper-Virus To Improve Transduction Efficiency of Conventional AAV Vectors In Vitro and In Vivo*  
Li Zhong, Linyuan Chen, Yanjun Li, Keyun Qing, Rebecca J. Chan, Mervin C. Yoder, Koppal S. Rao, Arun Srivastava

**343 3:15 PM**

*Characterization of Delivery Routes of AAV Vectors for Neonatal Gene Transfer*

Tsuyoshi Ogura, Hiroaki Mizukami, Jun Mimuro, Takashi Okada, Hiromi Hamada, Akihiro Kume, Hiroyuki Yoshikawa, Yoichi Sakata, Keiya Ozawa

**344 3:30 PM**

*Recombinant Adeno-Associated Virus 2 Suicide Vectors for the Treatment of Human Sarcomas and Mesotheliomas*

Marlon R. Veldwijk, Simone Berlinghoff, Stephanie Laufs, Frederik Wenz, Stefan Fruehauf, W. Jens Zeller

**345 3:45 PM**

*Sensitive Molecular Detection of Various Stable Episomal Concatemeric and Integrated Forms of rAAV2 after Long-Term Expression In Vivo*

Karim Zaoui, Manfred Schmidt, Denise E. Sabatino, Fayaz R. Khazi, Hanno Glimm, Junwei Sun, Katherine A. High, Christof von Kalle

**346 4:00 PM**

*Characterization of Adeno-Associated Virus Sequences in Human Tissues*

Chun-Liang Chen, Ryan L. Jensen, Bruce C. Schnepf, K. Reed Clark, Philip R. Johnson

### Cancer Immunotherapy

Room 208 A-D

**347 1:45 PM**

*Pharmacological Regulation of Dendritic Cell Activation: Enhancing Cancer Vaccine Efficacy*  
Brent A. Hanks, Jianghong Jiang, David M. Spencer

**348 2:00 PM**

*IL-21 and IL-15 Gene Therapy Induces Powerful Antitumor Effect Against Head and Neck Squamous Cell Carcinoma*

Hiroshi Nakano, Tsunao Kishida, Hidetsugu Asada, Takashi Shinomiya, Jiro Imanishi, Yasuo Hisa, Osamu Mazda

**349 2:15 PM**

*GM-CSF / IL-2 Fusion Transgene Is More Potent Than GM-CSF and IL-2 in Combination and Is Dependent upon CD8 and NK Cells for Antitumor Effect*  
John Stagg, Nathaniel Bouganim, Jacques Galipeau

**350 2:30 PM**

*Gene Transfer of Modified Idiotype into Dendritic Cells for Immunotherapy of Non-Hodgkin Lymphomas*  
Sara Muraro, Attilio Bondanza, Matteo Bellone, Philip Greenberg, Chiara Bonini

**351 2:45 PM**

*Enhanced Therapeutic Efficacy of a Cytokine Expressing Oncolytic Herpes Simplex Virus Against Prostate Cancer*

Susan Varghese, Samuel D. Rabkin, Wenzheng Wang, Robert L. Martuza

**352 3:00 PM**

*Intentional Killing of Normal Melanocytes by Gene Transfer Generates Curative Anti Melanoma Immunity without Obligate Autoimmunity*

Gregory Daniels, Luis Sanchez-Perez, Timothy Kottke, Rosa M. Diaz, Jill Thompson, Mayoi Lai, Michael Gough, Mahzuz Karim, Andrew Bushell, Heung Chong, Alan Melcher, Kevin Harrington, Richard G. Vile

**353 3:15 PM**

*In a Model of Tumor Dormancy, Long-Term Persistent Leukemic Cells Have Increased B7-H1 and B7.1 Expression and Resist CTL-Mediated Lysis*  
Aurore Saudemont, Bruno Quesnel

**354 3:30 PM**

*CD8 Immunomagnetic Selection and Interleukin-15 (IL-15) Facilitate the Isolation of Human CD8+ Cytotoxic T-Lymphocytes (CTL) Genetically Engineered To Express an Anti-CD20 Chimeric T-Cell Receptor (cTCR)*  
Eric Y. Chen, Oliver W. Press, Michael C. Jensen

**355 3:45 PM**

*Specific Lysis of Multiple Myeloma Cells by T-Cells Engineered To Express a Chimeric Immune Receptor Targeting WUE-Antigen*

Max S. Topp, Horst D. Hummel, Gabriele Kuntz, Christina Neff, Hermann Einsele

**356 4:00 PM**

*IL-12 Transduced Bone Marrow Cells Home to Bone and Generate Anti-Metastatic Activities in a Mouse Prostate Cancer Model*

Hongyu Wang, Guang Yang, Terry L. Timme, Tetsuo Fujita, Koji Naruishi, Xiaorong Ji, Malcolm K. Brenner, Dov Kadmon, Timothy C. Thompson

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

#### Molecular Conjugates

Auditorium Room 1

**357 1:45 PM**

*Selective Depletion of Kupffer Cells Leads to Enhanced Transgene Expression in Rat Liver Following Intraductal Infusion of Naked DNA and DNA Nanoparticles*

Hui Dai, Xuan Jiang, Weiseng Lim, Xuesong Jiang, Yong Chen, Hai-Quan Mao, Kam W. Leong

**358 2:00 PM**

*Route of Administration of sec-R Targeted Compacted DNA Influences Both the Site of Expression and Host Immune Response to the Vector*

Assem G. Ziady, John Kim, Joseph Colla, Pamela B. Davis

**359 2:15 PM**

*Enhanced Gene Delivery by a Novel Tumor-Specific Vector Containing Peptide and Nucleic Acid Based Nuclear Translocation Signals*

Stanley Saamoah-Moffatt, Sandra Wiegle, Richard J. Cristiana

**360 2:30 PM**

*Long-Term Gene Transfer and Expression in Xenografted Human Glioblastoma by Non-Viral Gene Transfer with the Sleeping Beauty Transposon*

John R. Ohlfest, Scott Perkinson, Paul Lobitz, Diane Hasz, David A. Largaespada

**361 2:45 PM**

*Reduction of Polyethylenimine Buffering Capacity Enhances In Vitro Gene Delivery Activity*

M. Laird Forrest, Nathan Gabrielson, Daniel W. Pack

**362 3:00 PM**

*Novel Synthetic Poly(hydroxylamidoamine)s Facilitate Efficient and Nontoxic Gene Delivery with Mammalian Cells*

Theresa M. Reineke, Yemin Liu, Laura Wenning

**363 3:15 PM**

*Uptake and Intracellular Trafficking of Stabilized and Unstabilized Gene Delivery Particles*

Swaroop Mishra, Paul Webster, Mark E. Davis

**364 3:30 PM**

*The Proteasome Metabolizes Non-Viral Gene Delivery Systems*

Ji-seon Kim, Kevin G. Rice

**Friday, June 4**

**365 3:45 PM**

*Cell Membrane Lipid Rafts Mediate Caveolar Endocytosis of HIV-1 Tat Fusion Proteins*

Antonio Fittipaldi, Silvia Agostini, Aldo Ferrari, Caterina Arcangeli, Vittorio Pellegrini, Fabio Beltram, Mauro Giacca

#### Infectious Diseases and Vaccines

Auditorium Room 3

**366 1:45 PM**

*Negative Feedback Inhibition of HIV-1: A Novel HIV-1 LTR-hsp70 Fusion Promoter Directs HIV-1 Inducible siRNA Expression*

Hoshang J. Unwalla, James D. Kim, Haitang Li, John J. Rossi

**367 2:00 PM**

*Development of Lentiviral Vectors Carrying shRNA, Ribozyme and RNA Decoy for the Inhibition of HIV-1 Infection*

Ming-Jie Li, James Kim, Shirley Li, John Zaia, Jiing-Kuan Yee, John J. Rossi

**368 2:15 PM**

*Pseudotyping HIV Vector with the Spike Envelope Protein of SARS-CoV for Studying Viral Tropism, Immunology, and Gene Therapy Applications*

Gary P. Kobinger, Heinz Feldmann, Gregory P. Schumer, James M. Wilson

**369 2:30 PM**

*Selective Expansion of Human T Lymphocytes Transduced by HIV Vectors Containing IMPDH2 and Anti-HIV siRNA Genes*

Priscilla Yam, Ying Yu, Jerry Wu, Michael C. Jensen, John A. Zaia, Jiing-Kuan Yee

**370 2:45 PM**

*Potent In Vivo Inhibition of HIV-1 Infection in Thy/Liv-SCID-Hu Mice after In Vivo Treatment with a Combination of SV40-Derived Vectors Targeting CCR5 Expression*

Mahe什 Patel, Pierre Cordelier, David S. Strayer, Harris Goldstein

**371 3:00 PM**

*Complete Inhibition of Hepatitis B Virus Gene Expression In Vivo with Short Hairpin RNA Expressed from a Novel Double-Stranded, Bi-Cistronic Adeno-Associated Virus Pseudotype 8 Vector*

Dirk Grimm, Konrad L. Streetz, Theresa A. Storm, Hiroyuki Nakai, Anton P. McCaffrey, Zan Huang, Felix H. Salazar, Patricia L. Marion, Mark A. Kay

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

**372 3:15 PM**

*Passive Immunotherapy for Anthrax Mediated by an Adenovirus Expressing Anti-Protective Antigen Single Chain Antibody*

Kazuhiko Kasuya, Julie L. Boyer, Yadi Tan, D. Olivier Alipui, Neil R. Hackett, Ronald G. Crystal

**373 3:30 PM**

*Simian Adenoviral Vector Based-Vaccine Fully Protect Against Ebola Virus Even in the Presence of Pre-Existing Immunity to Human Adenovirus*

Gary P. Kobinger, Heinz Feldmann, Yan Zhi, Gregory P. Schumer, Guangping Gao, Frederik Feldmann, Steven Jones, James M. Wilson

**374 3:45 PM**

*Development of a Genetic Vaccine Conferring Protective Immunity Against  $\alpha$ -Cobratoxin Following a Single Administration of an Adenovirus Vector Encoding a Modified, Non-Toxic Cobratoxin Variant*

Robert G. Pergolizzi, Rachel Dragos, Alexander Ropper, Andre Menez, Ronald G. Crystal

**375 4:00 PM**

*The Function of F12-Vif Mutant in the Context of APOBEC3G/Vif Model*

Giuliana Vallanti, Rossella Lupo, Maurizio Federico, Fulvio Mavilio, Chiara Bovolenta

### Gene Therapy for Musculoskeletal Diseases

Room 103 A-C

**376 1:45 PM**

*A Metabolically Responsive Insulin Transgene Delivered by AAV2/8 Pseudotyped, Self-Complementary AAV (SC-AAV2/8) Controls Glycemia in STZ-Diabetic Mice*

Mirek Kozlowski, Dorota Lyszcowicz, Adam G. Campbell, Darin E. Olson, Janet E. Rubin, Peter M. Thule

**377 2:00 PM**

*Expression of Acid  $\alpha$ -Glucosidase in Nonhuman Primate Diaphragm and Peritoneum after Direct In Utero Delivery of Recombinant Adeno-Associated Virus Type 1*

Mary B. Rucker, Alice F. Tarantal, Thomas Conlon, Terence R. Flotte, Barry J. Byrne

**378 2:15 PM**

*An AAV Vector-Mediated Micro-Dystrophin Expression in Relatively Small Percentage of Dystrophin-Deficient mdx Myofibers Still Improved the mdx Phenotype through Compensatory Hypertrophy*

Madoka Ikemoto, Madoka Yoshimura, Miki Sakamoto, Yasushi Mochizuki, Katsutoshi Yuasa, Toshifumi Yokota, Yuko Miyagoe-Suzuki, Shin'ichi Takeda

**379 2:30 PM**

*Myogenic Differentiation of Multipotent Adult Progenitor Cells Derived from Bone Marrow and Muscle Tissue*

Morayma Reyes, Sheng Li, En Kimura, Leonard Meuse, Jeffrey Chamberlain

**380 2:45 PM**

*Dedifferentiation of Primary Murine Myoblasts Induced by Ectopic Expression of Msx1*

Qiang Liu, Wen Liu, Paul Allen, Yaming Wang

**381 3:00 PM**

*Self-Cleaving Multimeric Hammerhead Ribozymes Abolish Mutant Collagen Transcripts In Cellulo and Exhibit Activity In Vivo*

Belinda E. Peace, Jane B. Florer, George Y. Wu, Yoel Smicum, Michael W. Kilpatrick, Petros Tsipouras, Richard J. Wenstrup

**382 3:15 PM**

*In Utero Intramuscular Delivery of a High-Capacity Adenoviral Vector Carrying a Full-Length Murine Dystrophin cDNA Provides Functional Benefit and Reassembly of the Dystrophin-Glycoprotein Complex in Hind Limb Muscles of mdx Mice*

Daniel P. Reay, Roberto Bilbao, Eric Wu, Terry L. O'Day, Jon F. Watchko, Paula R. Clemens

**383 3:30 PM**

*Cloning of the Minimal Functional Domain of Human Lim Mineralization Protein-3 Able To Induce Bone Mineralization: In Vitro and In Vivo Study*

Enrico Pola, Wanda Lattanzi, Wentao Go, Huijie Sun, Kaori Okada, Andrea Gambotto, Carlo A. Logroscino, Paul D. Robbins

**384 3:45 PM**

*Stem Cells Expressing SMAD 8: A Platform for Tendon Regeneration*

Gadi Pelled, Andrea Hoffman, Gadi Turgeman, Peter Eberle, Yoram Zilberman, Hadassah Shinhar, Keren Keinan-Adamsky, Andreas Winkel, Gil Navon, Gerhard Gross, Dan Gazit

**Friday, June 4**

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Friday, June 4

**385 4:00 PM**

*Noggin Gene-Based Therapy Inhibits Cranial Defect Healing in Mice*

Gregory M. Cooper, Hairong Peng, Arvydas Usas, Mark P. Mooney, Johnny Huard

### Cardiovascular Gene Therapy

Room 102 A-C

**386 1:45 PM**

*Intra-Arterial Adenoviral Mediated  $\beta$ 2Adrenergic Receptor (AR) Gene Delivery Induces Neoangiogenesis in Rat Ischemic Hindlimb*

Guido Iaccarino, Vincenzo Cerullo, Michele Ciccarelli, Gennaro Galasso, Daniela Sorriento, Walter J. Koch, Federico Piscione, Lucio Pastore, Francesco Salvatore, Bruno Trimarco

**387 2:00 PM**

*The Size of Sinusoidal Fenestrae Is a Critical Determinant of Hepatocyte Transduction after Adenoviral Gene Transfer*

Joke Lievens, Jan Snoeys, Katrien Vekemans, Sophie Van Linthout, Ronald de Zanger, Désiré Collen, Eddie Wisse, Bart De Geest

**388 2:15 PM**

*Protection of End-Organ Damage in Stroke-Prone Spontaneously Hypertensive Rats by Intramuscular Injection of AAV Vector Expressing Interleukin-10*

Tatsuya Nomoto, Takashi Okada, Kuniko Shimazaki, Toru Yoshioka, Mutsuko Sarukawa, Koichi Takeuchi, Hiroaki Mizukami, Takashi Matsushita, Ken-ichiro Katsura, Keiji Yamamoto, Akihiro Kume, Uichi Ikeda, Shigeo Ookawara, Yasuo Katayama, Keiya Ozawa

**389 2:30 PM**

*Placental Growth Factor (PIGF) Gene Transfer to the Periadventitial Tissue*

Shalini Bhardwaj, Himadri Roy, Kari Alitalo, Seppo Yla-Hertuala

**390 2:45 PM**

*eNOS Gene Transfected Endothelial Cells Inhibit Smooth Muscle Cell Proliferation and Platelet Aggregation In Vitro*

Guanhua Xue, Jiwei Zhang, Hao Zhang, baigen Zhang, Minli Sun

**391 3:00 PM**

*Targeted Delivery of Adenovirus Expressing eNOS to the Endothelium In Vivo Lowers Blood Pressure in Hypertensive Rats*

William H. Miller, M. Julia Brosnan, Delyth Graham, Arlene J. Frater, Keith M. Channon, Sergei M. Danilov, Paul N. Reynolds, Andrew H. Baker, Anna F. Dominiczak

**392 3:15 PM**

*High Intrinsic Expression of Manganese Superoxide Dismutase Is a Critical Protective Mechanism Against Oxidative Stress in Human Endothelial Progenitor Cells*

Tongrong He, Timothy E. Peterson, Ekhson L. Holmuhamedov, Andre Terzic, Noel M. Caplice, Larry W. Oberley, Zvonimir S. Katusic

**393 3:30 PM**

*rAAV-Mediated Apo A-1 Milano Gene Transfer Results in Significant Inhibition of Atherogenesis*

Sarah Hardy, Prediman K. Shah, Swapna Patel, Juliana Yano, Tripathi B. Rajavashisth, K. K. Wong, Saswati Chatterjee

**394 3:45 PM**

*Effective Focal, Homogeneous Gene Transfer after Coronary Infusion of Adenoviruses*

Tetsuo Sasano, Kan Kikuchi, Amy McDonald, Kevin Mills, Leah Peller, Kevin Donahue

**395 4:00 PM**

*Beta-Blockers Restrict Maximum Levels of Transgene Expression Elicited by CMV Promoters in Vascular Smooth Muscle Cells*

Husein K. Salem, Paul A. Kingston, Anthony M. Heagerty

### Poster Session II

Hall D

### RNA Virus Vectors II

**396**

*Factors Affecting Retroviral Gene Transfer to Human CD34+ Cells*

Youngtae Hong, Seungshin Yu, Sujeong Kim, Joonggon Kim, Sunyoung Kim

**397**

*Transduction of Primary Human CD34+ Cells by Self-Inactivating FIV Vectors*

Charlene F. Barroga, Mehdi Gasmi, Olivera Marjanovic, Laura Innis, David J. Looney

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Friday, June 4

**398**

*Transduction of Human Hematopoietic Stem Cells and T-Lymphocytes with RD114-TR-Pseudotyped Lentiviral Vectors*

Francesca Di Nunzio, Bianca Piovani, Sabrina Cazzaniga, Chiara Bonini, Francois-Loic Cosset, Fulvio Mavilio, Anna Stornaiuolo

**399**

*Transduction of Hematopoietic Stem Cells Using Polymer-Concentrated Ecotropic Retrovirus Coupled with In Vivo Selection of Gene-Modified Cells*

Douglas W. McMillin, Natalia Landazuri, Bagirath Gangadharan, David Archer, Joseph Le Doux, H. Trent Spencer

**400**

*Transduction of Human Embryonic Stem Cells with Lentiviral Vectors: Variable Promoter Activity in Pluripotent Cells*

Andrew Hinton, Theodore Friedmann, Alberto Hayek

**401**

*Engineering Retroviral Vectors for Stem Cell Therapies - Overcoming the Effect of Gene Silencing*

Gary K. Lee, David V. Schaffer

**402**

*Retrovirus and Lentivirus Vectors Are Silenced in ES Cells by Dynamic Interplay of Multiple Epigenetic Modifications*

Shuyuan Yao, Tanya Sukonnik, Tara Kean, Rikki Bharadwaj, Peter Pasceri, James Ellis

**403**

*Selective Engraftment of Genetically Modified Hematopoietic Stem Cells by a Truncated Erythropoietin Receptor*

Fabrizia Urbinati, Alexis Grande, Francesco Lotti, Giulia Facchini, Giuliana Ferrari, Fulvio Mavilio

**404**

*Cloning and Expression of Canine O6-Methylguanine-DNA Methyltransferase Gene in Ex Vivo Cultured Cells*

Michail M. Zaboikin, Narasimhachar Srinivasakumar, Tatiana N. Zaboikina, Friedrich G. Schuening

**405**

*Efficient Lentivirus Vector-Mediated Transduction of Neonatal Porcine Islet Cell Clusters: A Powerful Tool for Xenotransplantation Research*

Samantha L. Ginn, Wayne J. Hawthorne, Anita T. Patel, Rebecca Stokes, Stacey N. Walters, Philip J. O'Connell, Peter B. Rowe, Ian E. Alexander

**406**

*Determination of the Most Efficient Vector System for Gene Transduction into Dendritic Cells (DC)*

Eigo Satoh, Hua Yan, Xiao-Kang Li, Wataru Sugiura, Naoki Yamamoto, Kenichi Teramoto, Shigeki Arii, Hiromitsu Kimura

**407**

*Comparison of Transgene Expression Driven from 4 Different Retroviral Long Terminal Repeats*

Sujeong Kim, Mi-Dong Kim, Seock Kang, Chang Wan Joo, Haesook An, Karim Lee, Sunyoung Kim, Seung Shin Yu

**408**

*Developing Lentiviral Vectors for Specific Gene Expression in CD4+ T Cells*

Kit L. Shaw, Karen Pepper, Denise Petersen, Donald B. Kohn

**409**

*In Vivo Comparison of Liver-Specific Enhancer-Promoter Constructs in the Context of Lentiviral Vectors*

Tatiana Khakhoulina, Tuan Huy Nguyen, Catherine Fux-Cametti, Didier Trono

**410**

*Multivalent siRNAs for Combinatorial HIV Gene Therapy*

Joseph S. Anderson, Akhil Banerjea, Ming-Jie Li, John Rossi, Ramesh Akkina

**411**

*Creation of Novel Lentiviral Vectors That Co-Package Anti-HIV Ribozymes with HIV-1*

Shuo Gu, James D. Kim, Gerhard Bauer

**412**

*Cyclosporine Inhibits the Induction of GFP Specific Immune Responses after Transplantation of Lentivirally Transduced GFP Expressing Hematopoietic Stem Cells in Dogs*

Karen Beagles, Laura Peterson, Tobias Neff, Hans-Peter Kiem

**413**

*Development and Characterization of Lentiviral Vectors for Hemophilia Gene Therapy*

Lieven Thorez, Michael Maris, Carol Miao, Nico Van Rooijen, Désiré Collen, Marinee Chuah, Thierry VandenDriessche

**414**

*Design of an HIV Vector for Site-Specific Gene Insertion*

Gilles Michel, Ying Yu, Jiing-Kuan Yee

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

**415**

*Conditional Expression of a Suicide Gene by the Telomere Reverse Transcriptase Promoter for Post-Therapeutic Deletion of Insertional Mutagenesis*  
Richard G. Painter, Nicholas A. Lanson, Zhengmin Jin, Frank Park, Guoshun Wang

**416**

*Transient Gene Expression by Non-Integrating Lentiviral (NIL) Vectors*

Sarah Nightingale, Roger P. Hollis, Catherine Yang, Ingrid Bahner, Karen A. Pepper, Donald B. Kohn

**417**

*Simultaneous Transfer of the p16INK4a and p21Waf1 Tumor Suppressor Genes Plus GFP Mediated by a Novel Polycistronic Retroviral Vector*  
Lia K. Muschellack, Bryan E. Strauss, Eugenia Costanzi-Strauss

### AAV Vectors: Production and Applications

**418**

*Adaptation of the Wave Bioreactor to Baculoviral Production of AAV Vectors: Scale-Up Considerations*  
Haifeng Chen, Shangzhen Zhou, Glenn P. Pierce, Peter Colosi

**419**

*Production of Recombinant Adeno-Associated Virus Type 5 in Insect Cells*  
Masashi Urabe, Takayo Nakakura, Keiya Ozawa, Robert M. Kotin

**420**

*Adaptation of Baculovirus System for Large-Scale Production of rAAV Vector Serotypes*  
Erik Kohlbrenner, George Aslanidi, David R. Knop, Oleg Gorbatyuk, Kevin Nash, Ronald J. Mandel, Nicholas Muzychka, Barry J. Burns, Richard O. Snyder, Sergei Zolotukhin

**421**

*Large-Scale Production of AAV and Adenovirus Vectors Using Active Gassing with Large Culture Vessel*  
Takashi Okada, Tatsuya Nomoto, Toru Yoshioka, Tsuyoshi Ogura, Takashi Matsushita, Hiroaki Mizukami, Akihiro Kume, Villy Nielsen, Keiya Ozawa

**422**

*Optimization of AAV Vector Production by Transient Transfection*  
Xiaofei Wang, Sooah Sohr, Floro Cataniag, Fraser Wright, Guang Qu

**423**

*Isolation of an Adeno-Associated Virus (AAV)-Specific Camelid-Derived Single Chain Antibody Fragment: A Novel Tool for Purification of AAV Vectors of Different Serotypes*

Paul P. A. Oranje, Peter Verheesen, Dennis Verbart, Yvonne Mijnsbergen, Hans J. W. Haard, Pim Hermans, Mark R. ten Haaft, Jaap Twisk, Paul A. Dijkhuizen, Wim T. J. M. C. Hermens, Janneke J. M. Meulenberg

**424**

*Development of a Scalable Column Chromatography-Based Purification Process To Manufacture Empty Capsid-Free AAV Vectors*  
Guang Qu, Xiaofei Wang, Jac Luna, Sooah Sohr, Floro Cataniag, Fraser J. Wright

**425**

*Formulation Development for AAV2 Vectors: Identification of Excipients That Inhibit Vector Aggregation*

J. Fraser Wright, Tannie Le, Hoc Nguyen, Jurg M. Sommer, Glenn Pierce, Guang Qu

**426**

*Novel Chromatographic Process for the Purification of Recombinant Adeno-Associated Virus Pseudotypes*  
Michael Moxley, Donald Fisher, Anthony P. Green, Timothy D. Kierstead

**427**

*Adipose Tissue as a Novel Target for In Vivo Gene Transfer Using Adeno-Associate Virus (AAV) Vectors*  
Hiroaki Mizukami, Jun Mimuro, Tsuyoshi Ogura, Takashi Okada, Akihiro Kume, Yoichi Sakata, Keiya Ozawa

**428**

*Adrenomedullin Gene Delivery Attenuates Renal Damage and Hypertension in Spontaneous Hypertension Rats*

Xin Wei, Chunxia Zhao, Jiangang Jiang, Zhenjun Liu, Daowen Wang

**429**

*Alpha 1 Antitrypsin Gene Delivery for Preventing Type 1 Diabetes in NOD Mice*  
Yuanqing Lu, Mei Tang, Clive Wasserfall, Young-Kook Choi, Thomas Gardmann, Martha Campbell-Thompson, Mark A. Atkinson, Sihong Song

**430**

*AAV-Microbead Conjugates for the Enhanced Delivery of AAV-Based Vectors*  
Samuel J. Farlow, Mark W. Pandori, Takeshi Sano

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

**431**

*Selective Upregulation of TGF $\beta$ 1 ACT in the Rat Heart To Induce Cardioprotection from Ischemia-Reperfusion Injury*

Kui Chen, Yong Liu, Dayuan Li, Paul L. Hermonat, Jawahar L. Mehta

**432**

*Safety of Administration of AAV2CUhCLN2, a Candidate Treatment for Late Infantile Neuronal Lipofuscinosis to the Brain of Rats and Non-Human Primates*

Neil R. Hackett\*, D. Eugene Redmond, Jr\*, Dolan Sondhi, E. Lela Giannaris, Elizabeth Vassallo, Stephen M. Kaminsky, Ronald G. Crystal,

\*Both authors contributed equally

**433**

*Micro-Utrophin as a Therapeutic Protein in rAAV Mediated Gene Therapy for Duchenne Muscular Dystrophy*

Michael J. Blankinship, Paul Gregorevic, Jeffrey S. Chamberlain

**434**

*Recombinant Adeno-Associated Virus 2 -Mediated Anti-Angiogenic Gene Therapy in a Mouse Model of Intraperitoneal Ovarian Cancer*

Tatyana Isayeva, Changchun Ren, Selvarangan Ponnazhagan

**435**

*Adeno-Associated Virus Vectors Are Able To Restore FALDH Deficiency in Cells of Sjögren-Larsson Syndrome*

Markus Braun-Falco, Stefanie Haug, Hildegard Buning

**436**

*Recombinant Adeno-Associated Virus Gene Therapy for Cob(I)Alamin Adenosyltransferase Deficiency*

Kirsten Elizabeth Erger, Thomas James Conlon, Dietrich Matern, Thomas A. Bobik, Terence R. Flotte

**437**

*Developing a Mouse Model of the Mitochondrial NARP Syndrome Using AAV-Ribozymes for ATP6*

Jennifer A. Bongorno, John Guy, Alfred S. Lewin

**438**

*In Vitro Studies towards the Development of a Recombinant Adeno-Associated Virus for the Treatment of Medium-Chain Acyl-CoA Dehydrogenase Deficiency*

J. Lawrence Merritt II, Dietrich Matern, Jerry Vockley, Joseph Huey, David B. Schowalter

**Friday, June 4**

**439**

*Neuronal Membrane Specific Binding Peptides Isolated Via Phage Display Biopanning Against Trisialogangliosides*

James K. Liu, Qingshan Teng, Diana Tanase, Mary Moses, Ajit Krishnaney, Nicholas M. Boulis

### Conditionally Replicating Adenovirus Vectors; Adenovirus Toxicity and Immune Response

**440**

*The Multidrug Resistant Phenotype in Tumor Cells Facilitates Adenoviral Replication: New Concepts for Oncolytic Vector Development*

Alexandra Bernshausen, Gabriel Glockzin, Klaus Mantwill, Hans-Dieter Royer, Karsten Juerchott, Bernd Gaensbacher, Per Sonne Holm

**441**

*Conditional Replication Competent Adenovirus Ad-mTERT-Δ19 Driven by Human Telomerase Promoter Replicates and Elicits Cytopathic Effect Specifically in Cancer Cells*

Eunhee Kim, Taeyoung Koo, Dong Hyun Ko, Yoon A. Kang, Joo-Hang Kim, Chae-Ok Yun

**442**

*Novel cox-2 Driven Infectivity Enhanced CRAds for Treatment of Ovarian Cancer*

Gerd J. Bauerschmitz, Anna Kanerva, Masato Yamamoto, Dirk Nettelbeck, Tanja Hakkarainen, Peter Dall, David T. Curiel, Akseli Hemminki

**443**

*Incorporating the Survivin Promoter in an Infectivity Enhanced CRAd-Analysis of Oncolysis in Five Different Cancer Cell Lines*

Zeng B. Zhu, Sharmila K. Makhija, Baogen Lu, Minghui Wang, Angel A. Rivera, Gene P. Siegal, Ronald D. Alvarez, David T. Curiel

**444**

*ADP-Overexpressing Adenovirus Elicits Enhanced Cytopathic Effect by Induction of Apoptosis*

Eunhee Kim, Taeyoung Koo, Dae-Bong Kang, Il-Kyu Choi, Joo-Hyuk Sohn, Chae-Ok Yun, Joo-Hang Kim

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

**445**

*CG5757, an Oncolytic Adenovirus for the Treatment of Retinoblastoma (Rb) Pathway-Defective and Telomerase-Positive Cancers*

Yuanhao Li, Natalie Nguyen, Derek Ko, Melinda VanRoey, Gail Colben, Neeraja Idamakanti, Orlena Tam, Trini Arroyo, PSeshidhar Reddy, Peter Working, De-Chao Yu

**446**

*A Rapid, Efficient and Feasible Method for Constructing Conditionally-Replicating Adenoviral Vectors That Specifically Target Cancer Cells with Multiple Factors*

Satoshi Nagano, Yohiteru Murofushi, Setsuro Komiya, Ken-ichiro Kosai

**447**

*Replication-Competent Oncolytic Adenovirus Vectors That Express TRAIL and Over-Express ADP*

Baoling Ying, Peter Krajcsi, Ann E. Tollefson, Jacqueline F. Spencer, Konstantin Doronin, Drew L. Lichtenstein, William S. M. Wold

**448**

*Armed Oncolytic Adenovirus with an Antiangiogenic Gene, sflt-1: A Novel Effective Antitumor Approach*

Zilai Zhang, Jinhui Wang, Weiguo Zou, Binghua Li, Zifei Pei, Jinfa Gu, Cheng Qian, Xinyuan Liu

**449**

*Evaluation of New Generation of Prostate Specific Replication Competent Adenoviruses with E1A-AR Chimera*

Ying Li, Chien-lun Chen, Aarup Kubal, Qinghua Xia, Ronald Rodriguez

**450**

*Anti-CD22 Immunolipoplexes Enhance Transduction of Adenovirus in B Lymphoma Cells Independent on Adenovirus' Receptor but Do Not Enhance the Cytotoxicity of Oncolytic Adenovirus*

Yaohe, Pen H., Jennelle, Gunnel, Shao-an, Beverly E. Griffin, Nick R. Lemoine

**451**

*A Novel Cell Cycle Dependent Conditionally-Replicating Adenovirus Vector for Targeting a Broad-Spectrum of Cancer*

Atique Ahmed, Jian Qiao, Jesus Moreno, Richard G. Vile

**452**

*Adenoviral Gene Therapy Using CRAd Targeted to Cells with Hyperactive Wnt/β-Catenin Signaling*

Phillip H. Abbosh, Kenneth P. Nephew

**453**

*Reproductive Safety Evaluation and Quantification of Adenoviral Vectors in the Mouse*

Gyu Seek Rhee, Hyun Joo Lee, Soon Sun Kim, Seung Jun Kwack, Rhee Da Lee, Ji Hyun Seok, Soo Yeong Chae, Jae Woo Kim, Young Hyuk Won, Seung Hoon Lee, Kui Lea Park

**454**

*Evaluation of Polyethylene Glycol (PEG) Modification of Adenoviral Vectors To Reduce Innate Immune Response and Increase Vector Persistence*

Hoyin S. Mok, Michael A. Barry

**455**

*Neutrophils Interact with Adenovirus Vectors Via the β2-Integrin CD11b*

Daniel A. Muruve, Matthew J. Cotter

**456**

*A Comparison of Adenoviral-Mediated Gene Transfer in Human and Mouse Dendritic Cells*

Brian J. Morrison, Yoshio Sakai, John E. Janik, John C. Morris

**457**

*Fibronectin Peptide Reduces the Immune Response to Adenovirus and Enhances Transgene Expression*

Takako Miyamae, Anthony D. Marinov, Sylvie Fabre, David C. Wilson, Tomoyuki Imagawa, Bruce C. Trapnell, Gregory P. Boivin, Raphael Hirsch

**458**

*Efficient Transduction of Human and Murine Cells and Circumvention of Preexisting Human Adenoviral Immunity by Porcine Adenoviral Vectors*

Dinesh Singh, Suresh K. Mittal

**459**

*Immune System Regulation of Transgene Expression in the Brain 1: Systemically Activated Immune Responses Silence Adenoviral Vector-Encoded Transgene Expression in the Brain through Both Non-Cytolytic, and Cytotoxic Mechanisms*

Carlos Barcia, Clare Thomas, Jeff Zirger, Weidong Xiong, Olivier Boyer, David Klatzmann, Maria G. Castro, Pedro R. Lowenstein

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

**460**

#### *Immunogenicity of E2b-Deleted Adenoviral Vectors Compared with First Generation Vectors*

Jie Mi, Shannon Barker, Leonard Meuse, Shanna Sawatzki, Dennis Hartigan-O'Connor, Jeffrey S. Chamberlain

**461**

#### *In Vivo Immunogenicity of Adenoviral Vectors with Improved Dendritic Cell Tropism*

Catherine Hsu, Megan M. Boysen, Phyllis D. Frosst, Dan J. Von Seggern

**462**

#### *Toxicity Related to Predosing with Adenoviral Vectors Carrying Lac-Z or Dominant Negative IKK2 Transgenes*

Viraj P. Mane, Racel Cela, Nicola Brunetti, Phil Ng, Brendan Lee

**463**

#### *Conditionally Replicative Adenovirus Expressing Degradation-Resistant p53 for Enhanced Oncolysis of MDM2 Overexpressing Cancers*

Victor W. van Beusechem, Petra B. van den Doel, Winald R. Gerritsen

**464**

#### *Severe Pulmonary Pathology after Intravenous Administration of Adenovirus Vectors in Cirrhotic Rats*

Jeffrey S. Smith, Jie Tian, Jay N. Lozier, Andrew P. Byrnes

### Lipid Mediated Gene Transfer

**465**

#### *Delivery of NF- $\kappa$ B Decoy Related Oligodeoxynucleotides Reduces Pro-Inflammatory Cytokine Responses Associated with Plasmid DNA/Lipid Mediated Gene Transfer to Murine Lungs*

Anusha Varathalingam, Ian A. Pringle, Seng H. Cheng, Deborah R. Gill, Stephen C. Hyde

**466**

#### *Downregulation of ATF2 Desensitizes Oral Squamous Cell Carcinoma to Apoptosis*

Sleiman N. Razzouk, Svetlana M. Dolgilevich, Dianne C. Duffey

**467**

#### *Cationic Liposome-Based Delivery of DNA Modulating Enzymes to Specifically Regulate Gene Expression in Carcinoma Derived Cell Lines*

Pamela M. J. McLaughlin, Monika Trzpis, Ieneke T. F. van der Gun, Martin C. Harmsen, Marcel H. J. Ruiters

**Friday, June 4**

**468**

#### *Tissue Factor as a Link between Wounding and Tissue Repair*

Jiang Chen, Michael Kasper, Katsumi Nakagawa, Lin Bai, Wu Gang, Youming Zhang, Per M. Humpert, Martin Andrassy, Thomas Luther, Stephan Schiekofer, Baosheng Chen, Michael Morcos, Andreas Hamann, David M. Stern, Peter P. Nawroth, Angelika Bierhaus

**469**

#### *Improved Transfection with Mixtures of Cationic Lipids: Application to Human Umbilical Artery Endothelial Cells (HUAEC)*

Li Wang, Robert C. MacDonald

**470**

#### *Enhanced Transposition Activities of Mutant Sleeping Beauty Transposases*

Bradley S. Fletcher, James Baus, Arne Heggestad

**471**

#### *Efficient Non-Viral Vectorisation of siRNA in the Brains of Newborn Mice*

Zahra Hassani, Gregory Lemkine, Patrick Erbacher, Carine Giovannangeli, Jean Paul Behr, Barbara A. Demeneix

**472**

#### *In Vitro and In Vivo Gene Expression Mediated by Cationic Sendai F/HN Virosomes*

Hong Sung Kim, Keun Sik Kim, Myung Min Choi, Ji Eun Lee, Yong Serk Park

**473**

#### *Magnetofection To Enhance Airway Gene Transfer*

Stefania Xenariou, Uta Griesenbach, Stefano Ferrari, Phillip Dean, Ron Scheule, Seng Cheng, Duncan Geddes, Christian Plank, Eric Alton

**474**

#### *The Barrier to Liposomal Transfection Is Enhanced by DNASES Lacking Lysosomal-Targetting Glycosylation*

Andrew Wilber, Gary Armour, Michael C. Schneider

**475**

#### *Anionic Polyethyleneglycol Lipids Added to Cationic Lipoplexes Increase Their Plasmatic Circulation Time*

Céline Nicolazzi, Nathalie Mignet, Nathalia Dela Figuera, Mamony Cadet, Raoul Torero Ibad, Johanne Seguin, Daniel Scherman, Michel Bessodes

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

**476**

*Human Mesenchymal Stem Cells Genetically Modified To Express High Levels of Erythropoietin through Transfer of an Artificial Chromosome Engineered Using the ACE System*  
Sandra Vanderbyl, Neil MacDonald, Tom Stodola, Adele Telenius, Sandra Stewart, Harry C. Ledebur, Jr., Carl F. Perez

**477**

*New Pyridinium Cationic Lipids for Gene Delivery Synthesized Via Pyrylium Salts*  
Marc A. Ilies, Betty H. Johnson, William A. Seitz, Edward L. Ezell, Aaron Miller, E. Brad Thompson, Alexandru T. Balaban

**478**

*Preinjection of Free DiC14-Amidine Liposome and Dexamethasone Synergistically Enhance the Intravenous Transfection Efficiency of Cationic Lipid/DNA Lipoplex*  
Caroline Lonez, Jean-Marie Ruysschaert, Abdelatif Elouahabi

**479**

*KLN-47, a Safe Lipophosphoramido Reagent, Efficiently Transfects (>100 kb) Large Plasmids into Epithelial Cells*  
Tristan Y. Montier, Laura Hyndman, A. Christopher Boyd, Catherine Payne, Ann Doherty, Pascal Delepine, Claude Ferec, David Porteous

**480**

*Magnetofection To Enhance Transfection Efficiency of Large Genomic Context Vectors In Vitro*  
Stefania Xenariou, Uta Griesenbach, Ramsay Bowden, Elizabeth Hillery, Ann Doherty, Duncan Geddes, David Porteous, Christian Plank, Eric Alton, A. Christopher Boyd

**481**

*Efficient Gene Transfer into Human Epithelial Cell Lines Using Glycosylated Cationic Carriers and Neutral Glycosylated Co-Lipids*  
Tristan Montier, Pascal Delepine, Thierry Benvegnu, Daniel Plusquellec, Claude Ferec

**482**

*Design and Production of Peptide Transferring Agents Using Recombinant TAT-Mu-NLS Sequences*  
Ramadas Y., Madhusudhana N. Rao, Vijaya Gopal

**Friday, June 4**

**483**

*Acoustically Active Liposomes for Oligonucleotide/Drug Encapsulation; Delivery Agents with Capacity for Ultrasound-Triggered Release*  
Shao-Ling Huang, David D. McPherson, Robert C. MacDanold

**484**

*Longevity of Expression Following Liposome-Mediated Gene Transfer*  
Wendy E. Walker, Andy D. Miller

**485**

*Marked Transfection Enhancement by the DPL (DNA/Peptide/Liposomes) Trimeric Complex*  
Jong-Gu Park, Ik-Jae Moon

**486**

*Plasmid DNA Constructs Expressing SARS-CoV Antigens Are Immunogenic in Mice and Rabbits*  
Adrian Vilalta, Michal Margalith, Tom Evans, David C. Kaslow, Peter Hobart

**487**

*Influence Surface Modification of Lipoplexes on the Pharmacokinetics of Non-Viral Vectors for Gene Delivery*  
Eric Perouzel, Annabelle Caussin, Kristina Ulrich, Andrew D. Miller, Charles Coutelle, Michael Keller, Michael Jorgensen

### Pulmonary Disease

**488**

*Adenovirus-Mediated Delivery of ADAM10, a Novel Candidate Gene for COPD, Results in Emphysematous Changes in the Mouse Lung*  
Hiroki Saitoh, Adriana Heguy, Timothy P. O'Connor, Ben-Gary Harvey, Philip L. Leopold, Neil R. Hackett, Adam Cieciuch, Ronald G. Crystal

**489**

*Comparison of AAV2 and AAV2/5 CFTR Vectors in the Lungs of Cynomolgus Macaques*  
Keith L. Munson, Laurie M. Tatalick, Carolina I. Smith, Anne C. Fischer, Pervin Anklesaria, Barrie J. Carter, William B. Guggino

**490**

*Long Term Expression and Biodistribution of HIV Vector Pseudotyped with Deletion Mutant of the Ebola Envelope Glycoprotein Following Instillation in Airway of Nonhuman Primates*  
Gregory P. Schumer, Gary P. Kobinger, Maria F. Medina, Daniel J. Weiner, James M. Wilson

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Friday, June 4

**491**

*Improved Apical Airway Epithelia Targeting Properties of FIV Vector Pseudotyped with Envelopes from LCMV*  
Douglas E. Dylla, Melissa A. Hickey, Daniel E. Michele, Beverly L. Davidson, Kevin P. Campbell, Paul B. McCray, Jr

**492**

*Characterization of the Apical Transduction of Well-Differentiated Mouse Tracheal Cultures by an Equine Lentivirus Pseudotyped with Avian Influenza Coat Proteins*

Tristan R. McKay, Manij Patel, Angela M. Giddings, Raymond J. Pickles, John C. Olsen

**493**

*Enhanced Transgene Transfer and Expression by an Endobronchially Delivered Pseudotyped AAV2/5 Vector*

Anne C. Fischer, Carolina I. Smith, Terence R. Flotte, William B. Guggino

**494**

*Topical Delivery of mRNA to the Murine Lung and Nasal Epithelium*

Hazel Painter, Stephanie G. Sumner-Jones, Seng H. Cheng, Steve C. Hyde, Deborah R. Gill

**495**

*Efficient In Vivo Transduction of Mouse Airway Epithelial Cells by the Simian Immunodeficiency Virus Vector Pseudotyped with Sendai Virus F and HN Proteins*

Hisao Shirohzu, Katsuhisa Mitomo, Toshiaki Tabata, Uta Griesenbach, Steve C. Hyde, Eric W. Alton, Yasuji Ueda, Mamoru Hasegawa

**496**

*Recombinant Sendai Virus Vector to Evaluate Vector Distribution in a Large Animal Model of Lung Gene Transfer*

Gerry McLachlan, Uta Griesenbach, Toshi Owaki, Liz Hillary, John Williams, Zhu Yafeng, Chris Boyd, Deborah Gill, Mamoru Hasegawa, Eric W. F. W Alton, David Collie

**497**

*Ciliated-Cell Specific Expression of a Transgene by a Self-Inactivating Murine Retrovirus in Human Bronchial Epithelial Cells*

Lawrence E. Ostrowski, Kerri A. Kendrick, Angela M. Giddings, John C. Olsen

**498**

*Targeted Gene Transfer to Lung Airway Epithelium Using Plasmid or Adenoviral Vectors Formulated with an Anti-Inflammatory Dexamethasone-Spermine Conjugate*

Amber Price, Maria Limberis, Jeffrey Gruneich, James M. Wilson, Scott L. Diamond

**499**

*Inhibition of Non-Small Cell Lung Cancer by Silencing EGFR with RNAi*

Ming Wei, M. Zhang, X. Zhang, J. Chen, L. Gao, J. Hu, Q. Y. Hong, M. West, C. X. Bai

**500**

*Local and Systemic Inhibition of Lung Tumor Growth after Liposome Mediated mda-7/IL-24 Gene Delivery*  
Rajagopal Ramesh, Isao Ito, Ryo Miyahara, Yuji Saito, Zheng Wu, Abner M. Mhashilkar, Deborah R. Wilson, Cynthia D. Branch, Sunil Chada, Jack A. Roth

**501**

*Aerosols of Compacted DNA Nanoparticles Retain Structural Integrity and Biological Activity*

Sharon M. Oette, Christopher R. Gedeon, Tomasz H. Kowalczyk, Tamara L. Fink, Susannah L. Hyatt, Robert C. Moen, Mark J. Cooper

**502**

*Formulation of Adenoviral Vectors with Inhibitors of Mucociliary Clearance Enhances Gene Transfer to Airway Epithelia In Vivo*

Patrick L. Sinn, Ankur Shah, Maureen D. Donovan, Paul B. McCray, Jr

**503**

*Gene Transfer Strategies for Pulmonary Hypertension*

Larry G. Johnson, Manij Patel, Miriam K. Vanhook, John C. Olsen

**504**

*Gene Transfection into Fetal Sheep Airway Epithelium Using Cationic BGTC/DOPE Liposomes*

Noufissa Oudrhiri, Dominique Luton, Pascal De Lagausie, Abderrahim Aissaoui, Michelle Hauchecorne, Michel Peuchmaur, Jean-Pierre Vigneron, Jean-Francois Oury, Yves Aigrain, Jean-Marie Lehn, Pierre Lehn

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

**505**

*Effective Transgene Expression in the Murine Lung Using Compacted DNA Nanoparticles Formulated with Plasmid DNAs of 5, 10, and 20 kbp*

Tamara L. Fink, Patrick J. Klepcyk, Sharon M. Oette, Christopher R. Gedeon, Susannah L. Hyatt, Tomasz H. Kowalczyk, Robert C. Moen, Mark J. Cooper

**506**

*LPS-Induced Lung Inflammation and Bronchial Hyperreactivity: Effects of Murine IL-10 When Injected in the Airways or Generated in the Circulation after Muscle Electroporation of Murine IL-10 Encoding Plasmid*

Virginie Deleuze, Jean Lefort, Michel-Francis Bureau, Daniel Scherman, B. Boris Vargaftig

**507**

*Down-Regulation of the Epithelial Sodium Channel ENaC through Antisense Oligonucleotides*

Uta Griesenbach, Sara Escudero Garcia, Chris Kitson, Raymond Farley, Charanjit Singh, Hazel Painter, Rebecca L. Smith, Ron Scheule, Seng Cheng, Mark Edbrooke, Steve Hyde, Duncan M. Geddes, Eric W. F. W. Alton

**508**

*AAV2 and AAV6 Serology in Normal and Cystic Fibrosis Populations*

Christine L. Halbert, A. Dusty Miller, Ron L. Gibson, Moira L. Aitken

**509**

*Non-Viral Gene Delivery in the Murine Nasal Epithelium by Hypotonic Shock*

Jerome L. Lemoine, Leaf Huang

**510**

*In Vivo Gene Expression of Aerosolized PEI Gene Vector Complexes Is Solvent-Dependent*

Carsten Rudolph, Aurora Ortiz, Ulrike Schillinger, Christian Plank, Joseph Rosenecker

**511**

*Improvement of Aerosol Delivery of Polyethylenimine (PEI)-Based Gene Vectors to the Lungs of Mice*

Carsten Rudolph, Aurora Ortiz, Ulrike Schillinger, Jurgen Jauernig, Christian Plank, Joseph Rosenecker

**512**

*Electroporation-Mediated Gene Transfer of Human Hepatocyte Growth Factor Reduces Bleomycin-Induced Pulmonary Fibrosis in Rats*

Amiq Gazdar, Patrick Fachinger, Jaroslaw Pierog, Steven Hyde, Mathias Gugger, Ralph Schmid, Thomas Geiser

**513**

*Prevention of Irradiation-Induced Organizing Alveolitis by Inhalation Delivery of Manganese Superoxide Dismutase-Plasmid/Liposome (MnSOD-PL) Complexes to Mouse Lung*

Matthew Carpenter, Anurag Agarwal, Suhua Nie, Lauren Hricisak, Michael W. Epperly, Joel S. Greenberger

**514**

*Potential of Bone Marrow Mesenchymal Stem Cells for Cystic Fibrosis Therapy*

Guoshun Wang, Bruce A. Bunnell, Richard G. Painter, Susan Tom, Nicholas A. Lanson, Jeffery L. Spees, Donna Bertucci, Daniel J. Weiss, Vincent G. Valentine, Darwin J. Prockop, Jay K. Kolls

### Central Nervous System II

**515**

*Mutation Independent Knockdown of Mutant mRNA by RNA Interference and Replacement by mRNA with Knockdown-Resistant Codons*

Rajendra Kumar-Singh, Erin Binkley, Siobhan Cashman

**516**

*Vestibular Hair Cell Regeneration and Re-Establishment of Balance Function Induced by Math1 Gene Delivery*

Hinrich Staecker, Mark Praetorius, Kim Baker, Douglas E. Brough

**517**

*Prevention of Onset of Parkinson's Disease by In Vivo Gene Transfer of Human Hepatocyte Growth Factor in Primate Model: A Model of Gene Therapy for Parkinson's Disease*

Hiromi Koike, Akihiko Ishida, Munehisa Shimamura, Naoyuki Sato, Naruya Tomita, Yasufumi Kaneda, Ryuichi Morishita

**518**

*Long-Term Lentiviral Vector-Mediated Transgene Expression in Neural Progenitor Cells Following Implantation into the Injured Rat Spinal Cord*

Bas Blits, Caroline V. Caperton, Brandon M. Kitay, Gaelle Corrales, Mary Bunge

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Friday, June 4

**519**

*Minimally Invasive Strategies for Administration of Genetically-Modified Glia to the Contused Spinal Cord*

Damien D. Pearse, Bas Blits, Mary B. Bunge, Patrick M. Wood, Andre R. Sanchez, Andres Hurtado, Enrique P. Lopez, Oudega Martin

**520**

*Canine Neural Progenitor Cells: Phenotypic Markers In Vitro and In Situ*

Raquel M. Walton, Deborah J. Watson, John H. Wolfe

**521**

*Helper-Dependent Adenovirus with Gene Switch Regulation May Provide an Optimal Vector of Therapeutic Gene Delivery for Treatment of Parkinson's Disease*

Q. Chen, Y. He, X. Q. Yuan, P. Ng, P. Yotnda, Y. Long, E. C. Lai, K. Yang

**522**

*Increased Expression of OCT4, SOX2 and FGF4 Genes Following Injection of Embryonic Stem Cell into Damaged Murine Brain*

Anand S. Srivastava, Steve Shenouda, Mohamed E. Moustafa, Ewa Carrier

**523**

*Inhibition of Retinal Neovascularization by Intraocular Gene Delivery of Soluble VEGF Receptors*

Abraham Scaria, Peter Pechan, Michael Lukason, Hillard Rubin, Samuel Wadsworth

**524**

*Adenovirus-Mediated Silencing of Huntingtin Expression by hsiRNA*

Bin Huang, Stefan Kochanek

**525**

*Therapy for Alzheimer's Disease by Neprilysin Gene Transfer*

Robert A. Marr, Edward Rockenstein, Mark S. Kindy, Louis B. Hersh, Fred H. Gage, Eliezer Masliah, Inder M. Verma

**526**

*A New Mouse Reporter Strain with Nuclear-Localized lacZ Expression Allows Reliable Detection of Transplanted and Donor-Derived Cells*

Pei-Rong Wang, Daniel G. Miller, George Vassilopoulos, David W. Russell

**527**

*CERE-110, an AAV2-Based Vector To Deliver NGF, Provides Trophic Activity to Basal Forebrain Cholinergic Neurons in Rats and Cynomolgous Monkeys*

Kathie M. Bishop, Katrin Hofer, Denise Rius, Arpesh Mehta, Shokry Lawandry, Lamar Brown, Brian Kruegel, Mehdi Gasmi, Mark Tuszyński, Raymond T. Bartus

**528**

*VEGF Gene Therapy with Retrogradely Transported Lentivirus Prolongs Survival in Mouse ALS Model*

Mimoun Azzouz, Thanh Le, Scott G. Ralph, Fraser Wilkes, Arthur H. Burghes, Susan M. Kingsman, Kyriacos A. Mitrophanous, Nicholas D. Mazarakis

**529**

*rAAV Mediated Parkin Expression Confers Some Protection in the Rat 6-OHDA Lesion Model*

Fredric P. Manfredsson, Alfred S. Lewin, Ronald J. Mandel

**530**

*Spinal Interleukin-10 (IL-10) Gene Therapy Using Viral and Non-Viral Vectors Controls Inflammatory- and Chronic Constriction Injury-Induced Pain States in Rats*

Erin D. Milligan, Raymond A. Chavez, Stephen J. Langer,

Evan M. Sloane, Maru Chacur, Julie L. Wieseler-Frank,

Matthew G. Frank, Terrence R. Flotte, Pedro Cruz, Leslie

Leinwand, Jennifer A. Shumilla, John R. Forsayeth,

Steven F. Maier, Linda R. Watkins

**531**

*Activation of VEGF by Engineered Zinc Finger*

*Transcriptional Activators: A Novel Therapeutic Approach to Diabetic Neuropathy*

Carolyn L. Dent, Gabriel Lau, Edward J. Rebar, Lei

Zhang, Yali Lee, Andrew C. Jamieson, Casey C. Case,

Tyler Martin, Philip D. Gregory

*Cervical Spinal Cord Delivery of a Lentiviral Vector in SOD-1 Transgenic Mice*

Diana Tanase, Qingshan Teng, Ajit A. Krishnaney, James K. Liu, Mary E. Garrity-Moses, Nicholas Mazarakis, Lucy E. Walmsley, Mimoun Azzouz, Nicholas M. Boulis

**533**

*In Vitro and In Vivo Expression of Modified Mouse Galactosylceramidase in the Twitcher Mouse Model of Krabbe Disease*

James Cronin, Xian-Yang Zhang, Jacob Reiser

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Friday, June 4

**534**

*Recombinant Adeno-Associated Virus Serotype 1 Vector-Mediated Transduction within the Murine Hippocampus*  
Chuansong Wang, Thomas J. Sferra

**535**

*Efficient Transduction of Rat Spinal Cord Cells In Vitro and In Vivo Using Neuron-Specific and Non-Specific Lentiviral Vectors*  
Tetsuya Nejime, Atsushi Miyano, Osamu Kakinohana, Martin Marsala

**536**

*Design and Production of an AAV2 Vector Encoding a Chimeric cDNA Allowing Efficient Secretion of Functional Human Neurturin from Human Cells*  
Anthony Ramirez, Junshan Hao, Eli Kethchum, Kevin Kwok, Dominick Vacante, Raymond Bartus, Mehdi Gasmi

**537**

*Further Characterization of Myo-Neurospheres*  
Simon P. Quenneville, Marie-Eve Ducharme, Joel Rousseau, Alain Garnier, Jacques P. Tremblay

**538**

*Functional Correction of A-T Cells by ATM Gene Expression Mediated by HSV Amplicon Vector after the Ionizing Radiation or under Oxidative Stress Condition*  
Ryan Manuszak, Rodney Shackelford, Charles J. Link, Suming Wang

**539**

*Biodistribution and Safety Profile of CERE-110 (AAV2-NGF) Delivered to the Rodent Brain*  
Mark Landers, Melissa Austin, Kevin Kwok, Eli Ketchum, Richard Lin, Leo Manlapaz, Arpesh Mehta, Javier Fernandez, Mehdi Gasmi, Dominick Vacante, Raymond T. Bartus, Kathie Bishop, Marie Printz

**540**

*Retroviral Mediated Gene Transfer of Hexb in Sandhoff Disease*  
Susan S. Tom, Bruce A. Bunnell

**541**

*Mechanisms of Adenovirus Vector Gene Delivery to the Inner Ear*  
Douglas E. Brough, Mark Praetorius, Chi Hsu, Kim Baker, Hinrick Staeker

### Infectious Diseases and Vaccines

**542**

*DNA Shuffling Generates Novel HIV-1 Envelope Variants That Can Induce Broadly Neutralizing Antibodies in Rabbits*  
Xiaohan Du, Li Xu, Wenge Zhang, Susan Tang, NayWei Soong, Michael B. Zwick, Dennis R. Burton, Robert G. Whalen

**543**

*Ad DNA Polymerase and Preterminal Protein (pTP) Genes Each Mediate Inhibition of HIV-1 Replication in Human Alveolar Macrophages*  
Robert J. Kaner, Francisco Santiago, Dmitri Igoukin, Jerome Schaack, Andrea Amalfitano, Ronald G. Crystal

**544**

*Strong, Long-Lasting Immune Responses Against Lentivirus Antigens Using rSV40 Vectors Carrying HIV Tat and Envelope*  
Hayley J. McKee, David S. Strayer

**545**

*Lentiviral Vector Mediated Gene Transfer of the Anti HIV Gene revM10 into Primitive Hematopoietic Cells for Gene Therapy of HIV Infection*  
Ingrid Bahner, Gerald Cho, Barbara Engels, Denise Peterson, Karen Pepper, Donald B. Kohn

**546**

*Optimization of Expression of the SIV-Specific 9456 Ribozyme by the tRNA<sub>Val</sub> PolIII Promoter within an MLV- or Lentiviral-Vector*  
S. E. Braun, FE Wong, AL Chesebro, B. Nornen, D. J. Looney, F. Wong-Staal, R. P. Johnson

**547**

*Antiviral RNA Interference Strategies That Resist HIV Escape*  
Joshua N. Leonard, David V. Schaffer

**548**

*Cyclophilin A as a Cellular Target for Gene Therapy of HIV-1*  
Harris S. Soifer, Reena Vishwanath, James Kim, Bret Heale, Lisa J. Scherer, John J. Rossi

**549**

*Nonconserved Sequence Motifs outside the Catalytic Core Improve the Trans-Splicing Efficiency of Anti-HIV Hammerhead Ribozymes*  
Marc Weinberg, John J. Rossi

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Friday, June 4

**550**

*Use of Plant Polysaccharides as Topically Applied Adjuvants for Enhancing Immune Responses Via Transdermal Gene Vaccination*

Jeng-Hwan Wang, Su-Jane Wang, Wen-Chi Wei, Chih-Chien Hsu, Chin-Jin Li, Ning-Sun Yang

**551**

*Enhanced Immune Stimulation and Antigen Presentation of i.m. Injected Plasmid DNA Vaccines by Electroporation*

Baowei Peng, Yonggang Zhao, Yuhong Xu

**552**

*Single-Chain Antibodies for Viral-Based Passive Immuno-Therapy for Prion Disease: Linear Epitope Mapping and Clearance from Thalamic Injection*

Charles A. Wuertzer, Mark A. Sullivan, Howard J. Federoff

**553**

*Silencing HBV Gene Expression with Pol II and Pol III Promoter-Derived Short Hairpin RNA*

Sergio Carmona, Abdullah Ely, Naazneen Molla, Marc Weinberg, Patrick Arbuthnot

**554**

*A Candidate SARS-Associated Coronavirus Vaccine Elicits Broad Immunity in Monkeys*

Wentao Gao, Azaibi Tamin, Adam Soloff, Leonardo D'Aiuto, Edward Nwanegbo, Paul D. Robbins, William J. Bellini, Simon Barratt-Boyes, Andrea Gambotto

**555**

*Langerhans Cells Modified by Gene Transfer of Full Length CMV pp65 Are More Potent Inducers of Cytolytic T Lymphocytes in a CD4-Dependent Manner Than Either CMV p495 Gene-Modified or p495 Peptide-Pulsed Langerhans Cells*

Jianda Yuan, Jean-Baptiste Latouche, Joanna Hodges, Glenn Heller, Isabelle Riviere, Michel Sadelain, James W. Young

**556**

*Toward the Development of Electroporation for Delivery of DNA Vaccines to Humans*

Dietmar Rabussay, Georg Widera, Lei Zhang, Gillis R. Otten, Barbara Doe, Mary Schaefer, Hong Liu, Jeffrey B. Ulmer

**557**

*Co-Administration of an Adenovirus Encoding the B Cell Stimulating Factor BAFF with Heat-Inactivated Pseudomonas aeruginosa Leads to Increased Anti-pseudomonal Humoral Immunity*

Christine Tertilt, Stefan Worgall, Michael C. Rivara, Ronald G. Crystal

**558**

*Anti-SARS Humoral and Cellular Immunity Evoked by an Adenovirus Vector Expressing Spike Glycoprotein from SARS Coronavirus*

Julie Boyer, Anja Krause, Jianping Qiu, Neil Hackett, Gary Kobinger, Yan Zhi, James M. Wilson, Ronald G. Crystal

**559**

*Generating Powerful Immune Responses Against Lentiviral Antigens: Using rSV40 Vector-Delivered Proinflammatory Cytokines to Potentiate Immune Responses vs. rSV40-Delivered Lentiviral Antigens*

Hayley J. McKee, David S. Strayer

**560**

*Induction of Prion Protein-Specific Antibody Responses Using PrP-Retroparticles*

Daphne Nikles, Patricia Bach, Klaus Boller, Christoph A. Merten, Fabio Montrasio, Ulrich Kalinke, Klaus Cichutek, Christian J. Buchholz

**561**

*Recombinant Adenovirus Carrying Spike Gene Induces Effective Humoral and Cellular Immunity Against SARS-CoV in Rats*

Ranyi Liu, Lizhe Wu, Bijun Huang, Jialin Huang, Wenlin Huang

**562**

*Human T-Cells Modified as Antigen Presenting Cells Are Superior to Professional APC in Stimulating T-Cell Responses*

Eleni C. Adamopoulou, Christian Sinzger, Gabriele Kuntz, Christina Neff, Hermann Einsele, Hans G. Rammensee, Max S. Topp

**563**

*Determinants of a Humoral Immune Response Against the Transgene Product after Adenoviral Gene Transfer*

Bart De Geest, Sophie Van Linthout, Jan Snoeys, Joke Lievens, Désiré Collen

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Friday, June 4

**564**

*T Cell Immunity Using Transgenic B Lymphocytes*  
Mara Gerloni, Marta Rizzi, Paola Castiglioni, Maurizio Zanetti

**565**

*Modulation of the Acute Phase Response by Recombinant Adeno-Associated Viral Expression of the IL-1 Type 1 Soluble Receptor*  
Gregory G. Simon, Barry J. Byrne, Mark A. Atkinson

**566**

*Inactivation of a Bacterial Quorum-Sensing Signal by Expression of Bacterial Autoinducer Inhibitor, *aiiA*, in Mammalian Cells*  
Egon A. Ozer, Joseph Zabner

**567**

*Effective Boosting of Protective Immunity Via Repeated Intramuscular Administration of a Human Adenovirus-Based Anthrax Vaccine*  
Yadi Tan, Julie L. Boyer, Neil R. Hackett, Ronald G. Crystal

**568**

*Comparison of Antigen-Specific Immune Responses Elicited by Recombinant Simian Adenoviral Vectors with Deletions in Either E1, or E1/E3, or E1/E4 Regions*  
Yan Zhi, Gary P. Kobinger, Heather Jordan, Guangping Gao, James M. Wilson

**569**

*Increased Antigen-Specific Humoral Response by Adenoviral Vector Prime and Adeno-Associated Viral Vector Boost*  
Yan Zhi, Lili Wang, Guangping Gao, James M. Wilson

**570**

*Genetic Vaccination Against *Yersinia pestis* Using Recombinant Adenovirus Vectors Expressing the V Antigen*  
Maria Chiuchiolo, Barry Shea, Julie L. Boyer, Neil R. Hackett, Ronald G. Crystal

### Tumor Vaccines

**571**

*Expression of IL-27 in Murine Carcinoma Cells Produces T Cell-Dependent and -Independent Antitumor Effects*  
Masatoshi Tagawa, Masako Chiyo, Osamu Shimozato, Ling Yu, Takehiko Fujisawa

**572**

*Augmentation of Immuno-Gene Therapy for Lung Cancer Using Chemotherapy*  
Eiji Suzuki, Veena Kapoor, Arminder S. Jassar, Andrew Haas, Larry R. Kaiser, Steven M. Albelda

**573**

*Immuno Gene Therapy of Feline Fibrosarcoma Using Intratumoral Magnetofection for Gene Delivery: Preliminary Results of a Veterinary Clinical Study*  
Ulrike Schillinger, Niels Kjaergaard, Kathrin Wiedmann, Anne Loecher, Stefanie Schlemmer, Bianca Schwarz, Tina Kempf, Johannes Hirschberger, Roberto Koestlin, Christian Plank, Bernd Gansbacher, Thomas Brill

**574**

*Systemic IL-12 Eliminates Pre-Established Leukemia in a Murine Model of Philadelphia Chromosome Positive Acute Lymphoblastic Leukemia*  
Tanja A. Gruber, Dianne C. Skelton, Karen Pepper, Denise Peterson, Donald B. Kohn

**575**

*Dendritic Cells Modified Using an Adenovirus Expressing Murine IL-12 Inhibits Development of Tumors in a Mouse Colon Cancer Model*  
Yoshio Sakai, Brian J. Morrison, John E. Janik, John C. Morris

**576**

*Antitumoral Effect Mediated by Two SFV-IL-12 Expression Vectors in a Murine Model of Colon Adenocarcinoma*  
Juan R. Rodriguez-Madoz, Erkuden Casales, Min Guan, Jesus Prieto, Cristian Smerdou

**577**

*MVA-MUC1-IL2 Vaccine Immunotherapy for Patients Post-Prostatectomy with Rising PSA and No Evident Metastases*  
Robert Dreicer, Frederick Ahman, Allan Pantuck, Caroline Beunkens, Nadine Bizourne, Bruce Acres, Teresa Maciejewska, Patrick Squiban

**578**

*Enhancement of GM-CSF Secreting Tumor Cell Vaccines by Docetaxel through Immune-Dependent and Independent Mechanisms*  
Rodney A. Prell, Lisa Sampson, Melinda VanRoey, Karin U. Jooss

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

**579**

*Generation of Potent Cellular Immune Responses Induced by Intradermal Delivery of Plasmid DNA Coexpressing Flt3 Ligand and GM-CSF*  
Yi-Te Yo, Chao-Liang Wu, Chi-Wen Lo, Ai-Li Shiau

**580**

*Inducible Expression of Interleukin-12 for the Treatment of Liver Metastases Using a High-Capacity Adenoviral Vector*  
Ruben Hernandez-Alcoceba, Itsaso Mauleon, Maider Zabala, Vijay Shankar, Stefan Kochanek, Jesus Prieto, Cheng Qian

**581**

*Enhanced Antitumor Effect of Interleukin-12 and B7.1 Expressing Oncolytic Adenovirus in Immunocompetent Mouse*  
Young-Sook Lee, Kyung-Ju Cho, Sungae Cho, Chae-Ok Yun, Joo-Hang Kim

**582**

*Detection of Enhanced Serum Antibody Production to Renal Cell Cancer Proteins and Identification of Serological Tumor Antigens in Patients Treated with the GM-CSF-Gene Transduced-Autologous Tumor Vaccines (GVAX®)*  
Takao Hashiguchi, Yukoh Nakazaki, Shirley Clift, Dale G. Ando, Shigetaka Asano, Kenzaburo Tani

**583**

*Efficient Non-Viral Gene Delivery to Primary, Leukemia B Cells by mRNA Transfection*  
Linhong Li, Cornell Allen, Rama Shivakumar, Jonathan M. Weiss, Joseph C. Fratantoni, Linda N. Liu

**584**

*Intratumoral Electrottransfer of Interleukin-12 Gene and Bleomycin Synergistically Induced Therapeutic Immunity and Suppressed Subcutaneous and Metastatic Melanomas in Mice*  
Tsunao Kishida, Hidetsugu Asada, Hiroshi Nakano, Kakei S. Yasutomi, Masaharu Shin-ya, Takashi Shinomiya, Feng-De Cui, Yasuo Hisa, Jiro Imanishi, Osam Mazda

**585**

*Anti-Angiogenic Drugs Synergize with a Membrane Macrophage Colony Stimulating Factor (mM-CSF) Based Tumor Vaccine to Therapeutically Treat Rats with an Established Intracranial Glioma*  
Martin R. Jadus, Edward W. B. Jeffes, Ramon Sanchez, Christina Delgado, Jian G. Zhang, Sakineh Khalaghizadeh, Tetyana Khomenko, Animesh Petkar, Chirag Shah, Brandon Knight, Seema Vohra, Reza Jahroudi, Terry Wepsic

**Friday, June 4**

**586**

*Gene Transfer of IP10, IP10+CD154 or IP10+IL12 cDNA Induces an Anti-Leukemic Immunity in a Murine Model of Acute Leukemia*  
Aurore Saudemont, Bruno Quesnel

**587**

*Further Advances in Immuno-Gene Therapy Against Human Papillomavirus by Adeno-Associated Virus Gene Transfer into Dendritic Cells*  
Yong Liu, Maurizio Chiriva-Internati, Hong You, Jawahar L. Mehta, Paul L. Hermonat

**588**

*A Central Role of RNA in Developing Therapeutic Vaccines for Cancer and Infectious Diseases*  
Eli Gilboa, Sandra Santulli-Marotto, Smita K. Nair, David Boczkowski, Yanbing Zhao

**589**

*Therapeutic Efficacy and Cellular Mechanism Mediated by Intratumoral and Intramuscular IL-12 Electrogenetherapy*  
Shulin Li, Francesca Mellieon, Robin Barret

### Cancer Suicide Gene Therapy

**590**

*SuperCD Enables Low Dose 5-FC Application for Effective Suicide Gene Therapy in a Rat Hepatoma Model Both In Vitro and In Vivo*  
Marie-Luise Lemken, Florian Graepler, Wolfgang A. Wybranietz, Ulrike Schmidt, Irina Smirnow, Christine D. Gross, Martin Spiegel, Andrea Schenk, Hansjoerg Graf, Ulrike A. Lauer, Michael Gregor, Sorin Armeanu, Michael Bitzer, Ulrich M. Lauer

**591**

*Comparison of Plant-TK/AZT Versus HSV-TK/Ganciclovir Enzyme/Prodrug Systems in a Human Glioma Cell Line*  
Mette Willer, Peter Kristoffersen, Zoran Gojkovic

**592**

*Transfection of Human Oral Cancer Cells Mediated by Transferrin-Associated Lipoplexes: Mechanisms of Cell Death Induced by Herpes Simplex Virus Thymidine Kinase/Ganciclovir Therapy*  
Silvia S. Neves, Sergio M. Simoes, Maria C. Pedroso de Lima

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

**593**

*Recombinant Measles Viruses Encoding Suicide Genes for Oncolytic Therapy*  
Christoph Springfield, von Messling Veronika, Frenzke Marie, Cattaneo Roberto

**594**

*Gene-Directed Enzyme Therapy with Carboxylesterase/CPT-11, but Not Thymidine Kinase/GCV, Enhances the Efficacy of the Conditionally Replicating Adenovirus AdΔ24*  
Dinja Oosterhoff, Herbert M. Pinedo, M. Adhiambo Witlox, Jan E. Carette, Winald R. Gerritsen, Victor W. van Beusechem

**595**

*Combination Tissue Specific SCC Antigen Promoter with CD/5-FC Gene Therapy Enhances Killing of Human Uterine Cervical Cancer Cells by Radiation*  
Keng-Fu Hsu, Cheng-Yang Chou, Helen H. W. Chen, Che-Hsin Lee, Ai-Li Shiau, Chao-Liang Wu

**596**

*Radiosensitization of Gliomas Using Lentiviral Vectors Expressing Antisense ATM*  
Teong L. Chuah, David G. Walker, Ming Q. Wei, Shaun P. Scott, Martin F. Lavin

**597**

*Combined Suicide/Cytokine Gene Therapy of Hepatocellular Carcinoma*  
Anna-Lisa Stefani, Luisa Barzon, Ignazio Castagliuolo, Maria Guido, Fabio Farinati, Giorgio Palù

**598**

*Interleukin-2 Enhanced the Anti-Tumor Effect of Adenoviral-Mediated HSV-tk Gene Therapy in a Murine Bladder Cancer Model*  
Shiji Terao, Toshiro Shirakawa, Nobuyuki Hinata, Sadao Kamidono, Akinobu Gotoh

**599**

*Low-Dose Etoposide Enhances Tumor-Specific Adenovirus-Mediated Cytosine Deaminase Gene Therapy through Induction of Integrin αvβ3 and αvβ5 Overexpression and Upregulation of Human Telomerase Reverse Transcriptase Promoter*  
Gia-Shing Shieh, Tzong-Shin Tzai, Yi-Te Yo, Ai-Li Shiau, Chao-Liang Wu

**600**

*Fusion of HSV-1 Thymidine Kinase to an 11 aa-peptide from the HIV-1 Tat Protein Promotes Intercellular Trafficking and Enhances Anti-Tumoral Activity In Vivo*  
Ennio Tasciotti, Mauro Giacca

**Friday, June 4**

**601**

*HIV-1 TAT Protein Transduction Domain-Mediated Enhancement of HSV-TK/Ganciclovir Suicide Gene Therapy*  
Outi Meriläinen, Tanja Hakkarainen, Tiina Pasanen, Riikka Pellinen, Jarmo Wahlfors

**602**

*Utilization of VP22 in Cancer Gene Therapy*  
Tanja Hakkarainen, Tiina Pasanen, Jarmo Wahlfors

**603**

*Prostate Tumor Regression Following Adenoviral Delivery of Flp Recombinase-Regulated Diphtheria Toxin Expression*  
Janet A. Sawicki, Weidan Peng

**604**

*Phase I/II Clinical Trial of Ad-OC-TK plus VAL for the Patients with Metastatic or Local Recurrent Prostate Cancer: Initial Experience in Japan*  
Toshiro Shirakawa, Nobuyuki Hinata, Shuji Terao, Katsumi Shigemura, Naonori Taniguchi, Kazuhito Fukushima, Khoji Sugimoto, Kazuro Sugimura, Masafumi Matsuo, Sakan Maeda, Sadao Kamidono, Chinghai Kao, Thomas A. Gardner, Leland W. K. Chung, Akinobu Gotoh

**605**

*Cytokine Profile Assessment of Mesothelioma Patients Treated with Escalating Doses of Intrapleurally Administered, Genetically Modified PA1STK Cells and a Twice Daily Regiment of Ganciclovir. A Phase I Clinical Trial*  
Patrick S. Byrne, Paul O. Schwarzenberger, Lynn Harrison, Judy Norton, Jay K. Kolls

**606**

*The NOD-SCID β2M Null Mouse Xenograft Model for Studying Activation, Expansion and GvHD Potential of Gene Transduced Human T Cells In Vivo*  
Jon E. Walker, Gerhard Bauer, Phillip Herrbrich, David Hess, Todd Meyerrose, Bruno Nervi, Michael Rettig, Jan A. Nolta, John F. DiPersio

**607**

*In Vitro and In Vivo Monitoring of 5-Fluorosytosine Metabolism with 19F-NMR Spectroscopy after Adenoviral Transduction of Cells with Bifunctional Yeast CDUPRT Fusion Gene*  
Vera Simonenko, Huandong Pageant, Du Fei, Thian C. Ng

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

#### Targeted Cancer Therapies II

**608**

*VAI Deleted Adenovirus as an Oncolytic Agent Targeting Human EBV-Associated Tumors*  
Yaohe Wang, Shao-an Xue, Gunnel Hallden, Jennelle Francis, Beverly E. Griffin, Nick R. Lemoine

**609**

*Highly Active, Cancer Specific Promoters for Gene Therapy of Small Cell Lung Cancer*  
Nina Pedersen, Mikkel W. Pedersen, Thomas T. Poulsen, Hans S. Poulsen

**610**

*Elucidating the Mechanism(s) for Tumor Specificity of Attenuated Measles Virus*  
Bambi D. Anderson, Takafumi Nakamura, Stephen J. Russell, Kah-Whye Peng

**611**

*Group I Intron-Mediated Trans-Splicing Revision of Mutant p53 RNA Transcripts*  
Daniel J. Catron, Takashi Watanabe, Kyung-Sook Shin, Eric M. Toloza, Seong-Wook Lee, Bruce A. Sullenger

**612**

*Effect of Adenovirus dl520 as Replicating Agent in Combination with Fractionated Radiotherapy in a Glioma Xenograft Model*  
Alexa Bieler, Alexandra Bernshausen, Sybille Staerk, Anka Gross, Bernd Gaensbacher, Per Sonne Holm

**613**

*Display of Different Protein Scaffolds on Envelope Glycoprotein of Avian Leukosis Virus*  
Pranay D. Khare, Stephen J. Russell, Mark J. Federspiel

**614**

*In Vivo and In Vitro Characterisation of Library Selected Matrix Metalloprotease Activatable Retroviruses*  
Irene Hartl, Richard M. Schneider, Yuansheng Sun, Stephen J. Russell, Christian J. Buchholz, Klaus Cichutek

**615**

*Feasibility of hTERT-Targeting Trans-Splicing Ribozyme in Tumor-Targeted Gene Therapy*  
Seung-Hee Hong, Yoon-Jong Lee, Haeng-Im Jung, Byung-Su Kwon, Seong-Wook Lee, Jin-Sook Jeong, Chang-Min Kim, In-Hoo Kim

**Friday, June 4**

**616**

*Retargeted Selectively Replicating Adenoviruses for the Therapy of Colon Cancer*  
Krisztian Gy Homicsko, Christophe Fuerer, Richard Iggo

**617**

*Prostate Cancer Radiosensitization by Targeting Poly (ADP-ribose) Polymerase*  
Li Zhang, Prafulla Gokhale, Rajshree Mewani, Bihua Li, Anatoly Dritschilo, Viatcheslav Soldatenkov

**618**

*Coxsackie-Adenovirus Receptor (CAR) Levels Can Be Increased in Human Xenografts Followed by Treatment with FK228, a Histone Deacetylase Inhibitor*  
Alian Aguila, Merrill E. Goldsmith, Santhi J. Gollapalli, Michael C. Alley, William W. Waud, Susan Bates, Tito Fojo

**619**

*Augmentation of Anti-Tumor Activity of a Recombinant Adeno-Associated Virus 2 Carcinoembryonic Antigen Vaccine with Plasmid Adjuvants*  
Selvarangan Ponnazhagan, Gandham Mahendra, Jose Lima, Wayne Aldrich, Connie Jenkins, Changchun Ren, Sanjay Kumar, Lisa Kallman, Theresa V. Strong, Denise R. Shaw, Pierre L. Triozzi

**620**

*A Longer Human Telomerase Reverse Transcriptase Promoter-Based Conditionally Replicative Adenovirus Shows Oncolysis with Less Toxicity for Ovarian Cancer Treatment*  
Yosuke Kawakami, Koichi Takayama, Masato Yamamoto, Dirk M. Nettelbeck, Minghui Wang, Mack N. Barnes, Gene P. Siegal, Ronald D. Alvarez, David T. Curiel, Jerry L. Blackwell

**621**

*Translational Restriction of Protein Expression of a Modified Suicide Gene and Cytotoxicity in Human Mammary Tumor Cells Expressing High Levels of the Translation Initiation Factor eIF4E*  
Don A. Sibley, James M. Mathis, Yoshi Odaka, B. J. Williams, Arrico DeBenedetti

**622**

*Discovery of Targeting Peptides for Selective Therapy of Medullary Thyroid Carcinoma*  
Miriam Böckmann, Matthias Drosten, Brigitte M. Pützer

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

**623**

*Transcriptional Targeting of Breast Cancer Employing the Heparanase Promoter*

Martina Breidenbach, Daniel T. Rein, Yuko Tsuruta, Minghui Wang, Isabell Herrmann, Ronald D. Alvarez, Paul N. Reynolds, David T. Curiel

**624**

*Effect of an Oncolytic HSV Mutant Expressing ICP34.5 under Control of a Nestin Promoter for Brain Tumor Therapy*

Hirokazu Kambara, Hideyuki Okano, E. Antonio Chiocca, Yoshinaga Saeki

**625**

*AAV Gene Therapy for Liver Metastatic Cancer*  
Ray A. Xu, Kevin Sun, Geoffrey W. Krissansen, Peter W. Fung, Sue Xu, Weidong Xiao, Juan Shi, Sheung-tat Fan

**626**

*A Novel Prostate-Specific Adenovirus for Gene Expression in Hormone-Sensitive and Hormone-Refractory Prostate Cancer Cells*  
Wing-Shing Cheng, Magnus Essand

**627**

*Widespread Intratumoral Viral Distribution Enables Local Control of Large Human Sarcoma Xenografts by Oncolytic Herpes Simplex Viruses*  
Mark A. Currier, Yoni H. Mahller, Lisa C. Adams, Brian Horsburgh, Frank Tufaro, Timothy P. Cripe

**628**

*An Oncolytic Adenovirus Armed with a GM-CSF Activating Designed Zinc-Finger Protein Transcription Factor: A Potential Cancer Vaccine Approach*  
Andreas Reik, Philip D. Gregory, Casey C. Case

**629**

*VEGF Promoter-Based Conditionally Replicative Adenoviruses Are Useful for the Treatment of Malignant Pleural Mesothelioma*  
Koichi Takayama, Akiko Ikegami, Hiroyuki Inoue, Lioudmila Kaliberova, Junji Uchino, David T. Curiel, Yoichi Nakanishi

### Gene Regulation: Targets

**630**

*Tamoxifen-Inducible Expansion of Murine Hematopoietic Stem Cells by Retroviral-Mediated Transfer of a HOXB4-ERT2 Fusion Protein*  
Yan Shou, Lilia Stepanova, Brian P. Sorrentino

**Friday, June 4**

**631**

*Modulation of Immune Response to Gene Therapy Derived Products by CD4+ CD25+ T Regulatory Cells*  
Andrea Annoni, Manuela Battaglia, Antonia Follenzi, Angelo Lombardo, Luigi Naldini, Maria-Grazia Roncarolo

**632**

*Retrovirally Transduced Muscle-Derived Stem Cells Facilitate Repair of Osteochondral Defects*  
Ryosuke Kuroda, Arvydas Usas, Hairong Peng, Masamitsu Sakamoto, Johnny Huard

**633**

*Dose-Dependent Gene Silencing by Lentivirus-Mediated RNA Interference (RNAi) in bcr-abl Positive Cells*  
Michaela Scherr, Karin Battmer, Arnold Ganser, Matthias Eder

**634**

*The Role of Ink4a-Arf Locus in Control of Hematopoietic Stem Cell Proliferation*  
Lilia Stepanova, Brian P. Sorrentino

**635**

*Macrophage Colony Stimulating Factor Expression in Retrovirally Transfected Cells Is Dependent upon the Adherence Status of the Target Cells*  
JianGang Zhang, Qinghong Dan, Timothy C. Fong, Christopher C. Williams, Maria D. Avina, Mehrdokht Tarbiyat-Boldaji, Sakineh Khalaghizadeh, Michael Irwin, Amy Nyugen, H. Terry Wepsic, Martin R. Jadus

**636**

*Reversal of Hypothalamic Diabetes Insipidus Using ElAV-Based Regulatable Lentiviral Vectors Expressing Arginine Vasopressin*  
Alison Bienemann, Pippa Radcliffe, Kyri Mitrophanous, Colin Glover, Nicholas Mazarakis, James Uney

**637**

*Intrathecal Electroporation of Doxycycline Regulated Proopiomelanocortin Gene for the Neuropathic Pain Therapy in CCI Rats*  
Chung-Ren Lin, Chien-Ming Wu, Ming-Wei Lin, Jiin-Tsuey Cheng

**638**

*Therapeutic Antisense-Induced Exon Skipping for Duchenne Muscular Dystrophy*  
Annemiek Aartsma-Rus, Anneke A. M. Janson, Wendy E. Kaman, Mattie Bremmer-Bout, Christa L. de Winter, Habte F. Teshale, Gert-Jan B. van Ommen, Johan T. den Dunnen, Judith C. T. van Deutekom

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Friday, June 4

**639**

*Gene Expression Analysis of LPS Stimulated THP-1 Cells Treated with Antisense IRAK1 Delivered Using a pH-Sensitive, Membrane Disruptive Polymer*  
Rachel E. Johns, Allan S. Hoffman, Patrick S. Stayton

**640**

*E1Bp-Specific CD8+ T Lymphocytes Are Highly Induced in the Liver and Spleen Eight Days after Adenoviral Infection*

Jian Chen, Sylvia A. McPherson, Allan Zajac, Hui-Chen Hsu, Qi Wu, Pingar Yang, Xin Xu, David T. Curiel, John D. Mountz

**641**

*Tetracycline-Inducible Human GDNF Expression Mediated by a Single AAV Vector*  
Abdelwahed Chtarto, Catherine Melas, David Blum, Jean-Marie Jaspar, Thierry Velu, Jacques Brotchi, Marc Levivier, Liliane Tenenbaum

**642**

*In Vitro Rescue of Radiosensitive Phenotype Using a Novel Human Artificial Chromosome (HAC) Vector Containing DNA-PKcs Gene*  
Akihiro Otsuki, Akihiro Kurimasa, Candice G. T. Tahimic, Motonobu Katoh, Mitsuo Oshimura

**643**

*Prolonged Expression of Human Alpha-1-Antitrypsin in C3H/Hej Mice with Helper-Dependent Adenoviral Vectors Irrespective of Tissue Distribution of Gene Expression*  
W. Michael McCormack, Racel G. Cela, Christian Clarke, Viraj Mane, Kiran Ubhayakar, Brendan H. Lee

**644**

*Decreased Generation and Cytotoxic Function of Adenovirus-Specific CD8 T Cells in Both CD28 and FasL Deficient Mice*  
Jian Chen, Sylvia A. McPherson, Allan Zajac, Hui-Chen Hsu, Qi Wu, Pingar Yang, David T. Curiel, John D. Mountz

**645**

*AAV Mediated Human Insulin Gene Expression in Liver of Diabetic Rats*  
Seonock Woo, Hyejoo Kim

**646**

*Covalently-Closed, Dumbbell-Shaped Double-Stranded DNA Constructs (dSLIM) with Specific Immunomodulatory Function*  
Nicole Hagner, Astrid Sander, Juliana Köck, Sven A. König-Merediz, Burghardt Wittig, Manuel Schmidt

**647**

*AP20817-Inducible Activation of a Chimeric Insulin Receptor Mimics Insulin Action in Hepatocytes Transduced with AAV*

Alberto Auricchio, Gabriella Cotugno, Pietro Formisano, Francesco Beguinot, Enrico M. Surace, Roy Pollock

**648**

*In-Vivo Adenovirus Mediated RNA Interference of Multidrug Resistance Protein-2 (MRP-2/ABCC2/cMOAT)*  
Iñigo Narvaiza, María Vera, Nerea Razquin, Mikel Zaratiegui, Jesús Prieto, Puri Fortes

**649**

*Repression of Vascular Endothelial Growth Factor-A Over-Expression in Glioblastoma Cells Using Engineered Zinc-Finger Transcription Factors*  
Andrew W. Snowden, Lei Zhang, Fyodor Urnov, Carolyn Dent, Andrew R. McNamara, Yann Jouvenot, Steve H. Zhang, Edward J. Rebar, Andrew C. Jamieson, Casey C. Case, Philip D. Gregory

**650**

*Hypoxia-Inducible Factor-1a DNA Induced Angiogenesis in Cerebral Ischemia Model in the Rat*  
Tatsuya Abe, Tsuyoshi Matsuda, Jian Liang Wu, Ryo Inoue, Minoru Fujiki, Hidenori Kobayashi

**651**

*The CD34 Antigen Molecular Domains Implied in Potential Homing and Engraftment of Hematopoietic Progenitors*  
Gurudutta U. Gangenahalli, Vimal K. Singh, Soumya Pati, Yogesh K. Verma, Pallavi Gupta, Rajat Kumar, Rakesh K. Sharma, Pratibha M. Luthra

**652**

*Analysis of the Genomic Stability of Herpes Simplex Virus Thymidine Kinase Variants in Retrovirally Transduced Lymphocytes*  
Vanessa L. Capacio, Yong Fan, Jolanta Stefanski, Alex H. Chang, Michel Sadelain, Isabelle Rivière

**653**

*Activation of an ATM/chk2-Dependent Pathway Is Required for HSV-1 Protein ICP0 Induced G2/M Cell Cycle Arrest*  
Han Li, Joseph C. Glorioso

**654**

*rAAV Mediated Ribozyme Delivery Targeting Mouse Rhodopsin*  
Marina Gorbatyuk, J. J. Pang, William W. Hauswirth, Alfred S. Lewin

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Directory

Friday, June 4

Saturday, June 5

**655**

*Gene Therapy for Inhibition of Corneal Neovascularization in a Mice Model with Endostatin Delivered by Adeno-Associated Viral (AAV) Vector*  
Li-Ju Lai, Jane Li, Ken-Kuo Lin, Zhoung Wang, Tong Zhu, Xiao Xiao

**656**

*Potential Regulatory Function of Dendritic Cells Overexpression Indoleamine 2,3-Dioxygenase*  
Naoko Funeshima, Masayuki Fujino, Yusuke Kitazawa, Torayuki Okuyama, Xiao-kang Li

**657**

*Immune Responses to Transgene Expression Targeted to Keratinocytes*  
Soosan Ghazizadeh, Lorne B. Taichman, Richard S. Kalish

**658**

*Different Regulation of Atrial and Ventricular HSP70 mRNA Expression during Warm Blood Cardioplegic Arrest in Rats*  
Ernö Remsey-Semmelweis, John G. Kral, Richard B. Wait, M. A. Q. Siddiqui

**659**

*Development of Designed Zinc Finger Protein Transcriptional Regulators for Use in Multi-Factor Therapeutic Angiogenesis*  
Edward J. Rebar, Brian Johnstone, Pei-Qi Liu, Andrew C. Jamieson, Yuxin Liang, Lei Zhang, Matthew C. Mendel, Lei Xu, Kaye Spratt, Casey C. Case

**660**

*Efficient Production of Transgenic Pigs by Lentiviral Gene Transfer into Preimplantation Embryos*  
Andreas Hofmann, Barbara Kessler, Sonja Ewerling, Marc Boelhauve, Gottfried Brem, Eckhard Wolf, Alexander Pfeifer

**661**

*Immune System Regulation of Transgene Expression in the Brain 3: Effects of the Immune Response on Transgene Expression from HIV-Derived Lentiviral Vectors Injected into the Rat Striatum*  
Pedro R. Lowenstein, Evelyn Abordo-Adesida, Carlos Barcia, Antonio Follenzi, Luigi Naldini, Maria G. Castro

Friday, June 4

Saturday, June 5

### Adenovirus Targeting

Room 205 A-D

**662 10:15 AM**

*Adenovirus Targeting Via Leucine Zipper Peptide Ligand Attachment*  
Joel N. Glasgow, Abner Mhashilkar, David T. Curiel, Nikolay Korokhov

**663 10:30 AM**

*Genetic Re-Targeting of Adenovirus Using a Hyperstable scFv Domain and an Affibody® Molecule Against Her2/neu*  
Leif Lindholm, Karin Andersson, Pierre Boulanger, Karolin Fryholm, Petra Henning, Rob Hoeben, Saw See Hong, Jenny Johannsson, Maria Magnusson, Susanna Myhre, Per-Åke Nygren, Elisabeth Pettersson, Stefan Ståhl, Jort Vellinga, Maria Wikman

**664 10:45 AM**

*Surface-Expressed Angiotensin-Converting Enzyme as a Novel Approach To Target Adenovirus to Dendritic Cells*  
Yosuke Kawakami, Sergei M. Danilov, Paul N. Reynolds, Jessica M. Grunda, Michael Passineau, Irina V. Balyasnikova, Joel N. Glasgow, Minghui Wang, Zen-Bian Zhu, Pierre L. Triozi, Wayne A. Aldrich, Jerry L. Blackwell, David T. Curiel

**665 11:00 AM**

*Enhanced Transgene Delivery to Activated Vascular Endothelial Cells In Vitro and In Vivo Using a Novel Strategy To Modify Adenovirus Tropism*  
Kenichi Ogawara, Marianne G. Rots, Robbert J. Kok, Dirk K. F. Meijer, Grietje Molema, Hidde J. Haisma

**666 11:15 AM**

*Effect of Adenovirus Serotype 5 Fiber and Penton Modifications on In Vivo Tropism in Rats*  
Campbell G. Nicol, Delyth Graham, William H. Miller, Theodore A. G. Smith, Stuart A. Nicklin, Susan C. Stevenson, Andrew H. Baker

**667 11:30 AM**

*A Novel Platform for Chemical Modification of Adenovirus Vector Capsids Based on Reactive Cysteines*  
Florian Kreppel, Judith Gackowski, Erika Schmidt, Stefan Kochanek

**668 11:45 AM**

*Affinity Immobilization of Adenoviral Vector on the Surface of Nanoparticles Augments Transduction*  
Ilia Fishbein, Michael Chorny, Ivan S. Alferiev, Origene Nyanguile, Robert J. Levy

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

**669 12:00 PM**

*Light-Induced Adenovirus Gene Transfer*  
Birgit Engesaeter, Anette Bonsted, Anders Hoegset,  
Kristian Berg, Gunhild Maelandsmoe

### Cancer Tumor Suppressor Gene and Apoptosis

Room 208 A-D

**670 10:15 AM**

*Intracellular ShigaToxin 1A1 Delivered by Adenovirus-Mediated Segmental Trans-Splicing Causes Apoptotic Cell Death In Vitro and In Vivo*  
Katsutoshi Nakayama, Robert G. Pergolizzi, Ronald G. Crystal

**671 10:30 AM**

*Overexpression of Manganese Superoxide Dismutase (MnSOD) Prevents Photodynamic Therapy (PDT)-Induced Porcine Esophageal Stricture*  
Anurag Agarwal, Michael W. Epperly, Yaron Perry, James D. Luketich, Joel S. Greenberger

**672 10:45 AM**

*Lentiviral Transduction of INK4A/ARF Protein Cooperates with Anti-Leukemic Agents To Induce Apoptosis of Leukemia Cells*  
Yuansong Bai, Yasushi Soda, Minghan Chen, Kiyoko Izawa, Seiichiro Kobayashi, Akira Tomonari, Jun Ooi, Fumitaka Nagamura, Satoshi Takahashi, Kaoru Uchimaru, Tohru Iseki, Hiroyuki Miyoshi, Tsuneo A. Takahashi, Kenzaburo Tani, Arinobu Tojo, Shigetaka Asano

**673 11:00 AM**

*Injection of Progenitor Cells from the Bone Marrow or Esophagus Prevents Irradiation-Induced Esophagitis*  
YunYun Niu, Michael W. Epperly, Hongmei Shen, Joel S. Greenberger

**674 11:15 AM**

*Improved Therapy of Prostate Carcinoma with TRAIL Gene Delivery Using Flt-1 Promoter Element in Combination with Ionizing Radiation*  
Sergey A. Kaliberov, Lyudmila N. Kaliberova, Donald J. Buchsbaum

**675 11:30 AM**

*Combination Therapy of Ad-md<sub>4</sub>7 and Herceptin Increases Cell Death in Her-2/Neu Positive Breast Cancer Cells*  
Sunil Chada, Abner Mhashikar, Tamra McKenzie, Yanna Liu, Stephen G. Swisher, Kelly Hunt

**Saturday, June 5**

**676 11:45 AM**

*A Role for Ceramide in AdGFPfasLTET Gene Therapy*  
James S. Norris, David H. Holman, Ahmed M. El-Zawahry, David A. Schwartz, Alicja Bielawska, Lina M. Obeid, Yusuf A. Hannun, Jian-Yun Dong

### Lipid Mediated Gene Transfer

Auditorium Room 1

**677 10:15 AM**

*Polycationic Liposome-Mediated Extracellular Superoxide Dismutase Gene Delivery Leads to High Levels of the Transgene Expression and Prevents Acute Liver Injury in Mice*  
Jian Wu, Li Liu, Roy D. Yen, Andrea Catana, Mark A. Zern, Michael H. Nantz, Stefan L. Marklund

**678 10:30 AM**

*Cytoskeleton as the Principal Determinant of the Size-Dependent DNA Mobility in Cytoplasm: A New Barrier for Non-Viral Gene Transfer*  
Emmanuel Dauty, Alan S. Verkman

**679 10:45 AM**

*Systemic Delivery and Tumor Gene Expression Using Polycation-Precondensed DNA Prepared as Stable Plasmid Lipid Particles*  
Lorne Palmer, Lloyd B. Jeffs, Kitty P. Y. Chan, Kevin McClintock, Cory Giesbrecht, James Heyes, Amy C. H. Lee, Ian MacLachlan

**680 11:00 AM**

*Lipid-Mediated HSP27 Delivery Improves Functional Recovery Following Moderate Spinal Cord Contusion Injury*  
Justine J. Overman, Leon L. Hall

**681 11:15 AM**

*Non-Viral DNA and mRNA Gene Delivery to the CNS Pre-Operatively for Neuroprotection and Following Neurotrauma*  
James G. Hecker, Alice F. Tarantal, Michael H. Nantz

**682 11:30 AM**

*New Histidylated Cationic Lipids for DNA- and mRNA-Based Lipofection*  
Valluripalli Vinod Kumar, Rajkumar Sunil Singh, Christine Gonçalves, Pierre Sandrin, Chantal Pichon, Patrick Midoux, Arabinda Chaudhuri

**683 11:45 AM**

*Cationic Mitochondriotropic Vesicles for DNA Delivery to Mitochondria*  
Volkmar Weissig, Gerard G. M. D'Souza

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

**684 12:00 PM**

*Expanded Parvoviral Replicons as Novel Nonviral Vectors for Cancer Gene Therapy*

Boris R. A. Blechacz, Jean Rommelaere, Christiane Dinsart, Brandenburger Annick, Russell J. Stephen

### What's Hot in the Lung: Recent Advances in Lung Gene Therapy

Auditorium Room 3

**685 10:15 AM**

*Epithelium-Specific Gene Therapy Vector Protects Cftr Knockout Mice from Acute Lung Infection*

David R. Koehler, Umadevi Sajjan, McKerlie Colin, Allan Coates, Keith Tanswell, Forstner F. Janet, Jim Hu

**686 10:30 AM**

*CAR-Expression Is Essential for Tumor Initiation in Lung Cancer Xenografts*

Min Qin, Brian Escudero, Ling Zhang, Sherven Sharma, Raj K. Batra

**687 10:45 AM**

*Parainfluenza Virus-Mediated Delivery of CFTR to Ciliated Cells Corrects the CF Bioelectrical Chloride Secretory Defect and Restores the Regulation of Airway Surface Liquid Depth and Mucus Transport to Cystic Fibrosis Airway Epithelium*

L. Zhang, B. Button, M. H. Skiadopoulos, Y. Dang, A. Bukreyev, S. E. Gabriel, P. L. Collins, R. J. Pickles

**688 11:00 AM**

*Pseudotyping FIV-Based Lentiviral Vectors with Baculovirus GP64 Confers Apical Entry into Airway Epithelia*

Patrick L. Sinn, Erin R. Burnight, Doug E. Dylla, Christina S. Firm, Melissa A. Hickey, Gary W. Blissard, Paul B. McCray, Jr

**689 11:15 AM**

*Protection Against Pulmonary Infection with *P. aeruginosa* Following Immunization with an Adenovirus Vector Expressing a *P. aeruginosa* OprF Epitope*

Stefan Worgall, Michael Rivara, Anja Krause, Neil R. Hackett, Peter W. Rovelink, Joseph T. Bruder, Thomas J. Wickham, Imi Kovacsdi, Ronald G. Crystal

**690 11:30 AM**

*Angiopoietin-1 Reduces Lung Edema and Mortality Induced by Bacterial Endotoxin*

Yao Qi Huang, Harald Sauthoff, Teona Pipiya, Shu Chen, William N. Rom, John G. Hay

**Saturday, June 5**

**691 11:45 AM**

*CFTR mRNA Is Expressed in Lungs of CFTR-KO Mice Following Administration of Adult Marrow Cells from Wild-Type Donor Mice*

Kaarlin K. Goncz, Roberto Loi, Travis Beckett, Robert Prenovitz, Daniel J. Weiss

**692 12:00 PM**

*A Novel AAV Vector for the Treatment of Cystic Fibrosis Airway Disease*

Maria Limberis, Guangping Gao, Daniel J. Weiner, Lili Wang, Peter Bell, James M. Wilson

### Inborn Errors of Metabolism: Lysosomal Storage and Other

Room 103 A-C

**693 10:15 AM**

*Retrovirally Transduced Bone Marrow Has Effect on Brain Pathology in Mouse Model of Mucopolysaccharidosis IIIB*

Yi Zheng, Sergey Ryazantsev, Kazuhiro Ohmi, Huizhi Zhao, Nora Rozengurt, Donald B. Kohn, Elizabeth F. Neufeld

**694 10:30 AM**

*Improved Therapeutic Efficacy Following AAV2/8-Mediated Expression of  $\alpha$ -Galactosidase A in Fabry Mice*

R. J. Ziegler, C. M. Barbon, C. Li, S. Bercury, M. Cherry, S. H. Cheng

**695 10:45 AM**

*Effect of Neonatal Hepatic Gene Therapy on Cardiovascular Disease, Hearing, and Visual Function in Mice with MPS I*

Yuli Liu, Hennig Anne, Kovacs Attila, Xu Lingfei, Fu Annabel, Chung Sarah, Ponder Katherine

**696 11:00 AM**

*Comparative Study of Lentiviral Vector-Mediated Gene Therapy in Neonatal and Young-Adult MPS I Mice by a Single Intravenous Injection*

Hiroshi Kobayashi, Denise Petersen, Denise Carbonaro, Dianne Skelton, Shundi Ge, Carolyn Luzko, Paula Cannon, Maya Otto Dussel, Michael Rosol, Rex Moats, Hiroyuki Shimada, Donald B. Kohn

**697 11:15 AM**

*Correction of Lysosomal Storage within the Brain of Adult MPS VII Mice Following Intrahepatic Administration of a Recombinant Adeno-Associated Virus Vector*

Kristin Backstrom, Chuansong Wang, Chiou-Miin Wang, Thomas J. Sferra

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Saturday, June 5

**698 11:30 AM**

*Gene Therapy of Murine GM1 Gangliosidosis by Genetically Modified Bone Marrow Hematopoietic Progenitor Cells*

Renata Sano, Alessandra Tessitore, Angela Ingrassia, Alessandra d'Azzo

**699 11:45 AM**

*Correction of Glycogen Storage Disease Type II (GSD II) with an Adeno-Associated Virus 8 (AAV2/8) Vector*  
Baodong Sun, Haoyue Zhang, Luis Franco, Ayn Schneider, Andrew Bird, Andrea Amalfitano, Y.-T. Chen, Dwight D. Koeberl

**700 12:00 PM**

*Targeted Delivery of a Recombinant Protein to Neurons and Astrocytes by Transport and Uptake Via the Low Density Lipoprotein (LDL) Receptor for Treatment of Neurodegenerative Disorders*  
Brian J. Spencer, Inder M. Verma

### Gene Transfer into Hematopoietic Stem/Progenitor Cells

Room 102 A-C

**701 10:15 AM**

*A SIN Lentiviral Vector That Allows In Vivo Drug-Selection of Hematopoietic Stem Cells yet Retains Erythroid-Specific Expression of the Ferrochelatase Gene and Results in Cure of Murine Protoporphoryria*  
Emmanuel Richard, Elodie Robert, Muriel Cario-Andre, Cecile Ged, Punam Malik, Hubert de Verneuil, Francois Moreau-Gaudry

**702 10:30 AM**

*Successful Correction of Human Beta Thalassemia Major Using a Self-Inactivating Lentiviral Vector*  
G. Puthenveetil, J. Scholes, D. Carbonell, N. Qureshi, P. Xia, L. Zeng, A. Hiti, J.-K. Yee, P. Malik

**703 10:45 AM**

*Efficient In Vivo Gene Marking of Hematopoietic Cells without Marrow Conditioning in Nonhuman Primates*  
Kyouji Ueda, Yasuji Ueda, Yutaka Hanazonno, Hiroaki Shibata, Naohide Ageyama, Takeyuki Nagashima, Toshiaki Tabata, Akihiro Kume, Susumu Ikebara, Keiji Terao, Keiya Ozawa, Mamoru Hasegawa

**704 11:00 AM**

*Ex Vivo and In Vivo Expansion of HoxB4-Transduced CD34+ Cells from Dogs, Baboons, Macaques and Humans*  
Xiao-Bing Zhang, Tobias Neff, Bobbie Thomasson, R. Keith Humphries, Hans-Peter Kiem

**705 11:15 AM**

*AMD3100-Mobilized CD34+ Cells Are Phenotypically Different and Better Targets for Retroviral Transduction Than G-CSF-Mobilized CD34+ Cells in Rhesus Macaques*  
Andre Laroche, Donald Orlic, Allen Krouse, Robert E. Donahue, Cynthia E. Dunbar, Peiman Hematti

**706 11:30 AM**

*Retrovirus Integration Site Distribution in Long-Term Gene-Corrected Hematopoiesis of the SCID-X1 Gene Therapy Clinical Trial*  
Manfred Schmidt, Jingjiong Hu, Salima Hacein-Bey-Abina, Alain Fischer, Manuela Wissler, Claudia Prinz, Hanno Glimm, Kerstin Schwarzwälder, Francoise LeDeist, Alexandrine Garrigue, Isabelle Andre-Schmutz, Marina Cavazzana-Calvo, Christof von Kalle

**707 11:45 AM**

*Retroviral Insertion Site Analysis in Rhesus Macaques Transplanted with CD34+ Hematopoietic Stem Cells Transduced with a Simian Immunodeficiency Virus-Based Lentiviral Vector*

Bum Kee Hong, Boris Calmels, Hideki Hanawa, John Gray, Robert E. Donahue, Derek A. Persons, Arthur W. Nienhuis, Peiman Hematti, Cynthia E. Dunbar

**708 12:00 PM**

*Retroviral Vector Integration into the Genome of Rhesus Macaque Long-Term Repopulating Cells*  
Cole Ferguson, Rima Adler, Stephanie Sellers, Manfred Schmidt, Christof von Kalle, Cynthia Dunbar, Boris Calmels

### Gene Regulation: Gene Modification and New Methods

Room 211 A-D

**709 10:15 AM**

*Gene Correction and Gene Knockouts in Human Somatic Cells Using Zinc Finger Nucleases*  
Matthew H. Porteus

**710 10:30 AM**

*Stem Cell Gene Therapy: Synergizing Improved Gene Repair/Modification & Targeted Integration of Transgenes to Transient In Vivo Topical Minigene Expression*  
Roger Bertolotti

**711 10:45 AM**

*Trans-Splicing Correction of CD40 Ligand Deficiency Resulting in Naturally Regulated Correction of a Murine Model of Hyper IgM X-Linked Immunodeficiency*  
Minoru Tahara, Robert G. Pergolizzi, Ronald G. Crystal

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Saturday, June 5

**712 11:00 AM**

*High Capacity Screen To Select Optimal Pre-trans-splicing Molecules for Trans-splicing Applications*

M. Puttaraju, Yanping Yang, Jonathan E. Spindler, Jun Wang, Colette A. Cote, Weng Ke, Garry P. Nolan, S. Gary Mansfield, Lloyd G. Mitchell, Edward Otto

**713 11:15 AM**

*Gene Correction Therapy Using Designed Zinc Finger-Based Endonucleases*

Michael C. Holmes, Fyodor D. Urnov, Ya-Li Lee, Jeffrey C. Miller, Philip D. Gregory

**714 11:30 AM**

*Structure-Based Engineering of the FokI DNA Cleavage Domain in the Context of a Chimeric Endonuclease: Towards Maximally Efficient "Gene Editing" Therapy*

Jeffrey C. Miller, Michael Holmes, Fyodor Urnov, Andrew C. Jamieson, Carl O. Pabo

**715 11:45 AM**

*Artificial Mammalian Gene Networks*

Beat P. Kramer, Wilfried Weber, Martin Fussenegger

**716 12:00 PM**

*The Human Norepinephrine Transporter and (11C)-mHED, a Novel Reporter Gene - Tracer Combination for PET Imaging of Gene Therapy*

Antoine M. J. Beerens, Anne Rixt Buursma, Marianne G. Rots, Aren van Waarde, Hidde J. Haisma, Erik F. J. de Vries

### Presidential Symposium

Auditorium

(Top 3 abstracts)

**717 3:15 PM**

*RNAi Therapy for Dominant Neurodegenerative Diseases*

Haibin Xia, Qinwen Mao, Steven L. Eliason, Nathan Kiewiet, Jaime Critchfield, Ines H. Martins, Scott Q. Harper, Xiaohua He, Robert M. Kotin, Huda Y. Zoghbi, Harry T. Orr, Henry L. Paulson, Beverly L. Davidson

**718 3:33 PM**

*Systemic Gene Transfer to Striated Muscles Using rAAV6 Vectors*

Paul Gregorevic, Michael J. Blankinship, James M. Allen, Leonard Meuse, Jay Han, Suzanne Oakley, Jeffrey S. Chamberlain

**719 3:51 PM**

*E1-Deleted Adenoviral Vectors Persist in Activated Lymphocytes and Cause Continued Transgene Expression and Stimulation of CD8+ Effector T Cells*

Julie Fitzgerald, Arturo Reyes-Sandoval, Nia Tatsis, Scott E. Hensley, Hildegund C. J. Ertl

### Poster Session III

Hall D

### RNA Virus Vectors III

**720**

*Tolerisation of Mice To Allow Repeated Administration of Recombinant Sendai Virus Vector to the Airways*

Uta Giesenbach, Rosemary Boyton, Luci Somerton, Sara Escudero Garcia, Stefano Ferrari, Yoshiyuki Owaki, Shu Tsugumine, Duncan M. Geddes, Mamoru Hasegawa, Danny Altmann, Eric W. F. W. Alton

**721**

*Proper Maturation of CFTR Protein Expressed by SeV Vector Was Proved by Using a GFP-CFTR Fusion Protein*

Hiroshi Ban, Makoto Inoue, Uta Giesenbach, Jun You, Steve C. Hyde, Eric W. F. W. Alton, Akihiro Iida, Mamoru Hasegawa

**722**

*SeV RNA Replicon Vector: An Efficient Cytoplasmic Expression System Derived from Sendai Virus Self-Replicating Ribonucleoprotein Complexes*

Akihiro Iida, Mamoru Hasegawa

**723**

*Sendai Virus Vectors without All of the Envelop-Related Genes: Successful Recovery and Characterization of the Three Genes (Matrix, Fusion and Hemagglutinin-Neuraminidase)-Deleted Vectors*

Makoto Inoue, Mariko Yoshizaki, Yumiko Tokusumi, Takashi Hironaka, Hiroshi Ban, Yoshiyuki Nagai, Akihiro Iida, Mamoru Hasegawa

**724**

*Engineering of Vesicular Stomatitis Virus-G Protein (VSV-G) To Retarget Recombinant Infectious Retroviral Particles*

Hanna S. Dreja, Marc Piechaczyk

**725**

*Engineering Enhanced Retroviral Vectors by Random Insertion of Functional Peptides*

Julie H. Yu, David V. Schaffer

# The American Society of Gene Therapy's 7th Annual Meeting final program

2004

## Abstract Directory

Saturday, June 5

**726**

*Ultrasound Enhances Retroviral-Mediated Gene Transfer*

Toshio Naka, Tsuyoshi Sakoda, Yoshio Fujioka, Takeshi Tsujino, Mitsumasa Ohyanagi

**727**

*Baculovirus GP64 Pseudotyped Lentiviral Vectors for Improved Hepatocyte Transduction*

David M. Markusic, Ronald Oude-Elferink, Jurgen Seppen

**728**

*Baculovirus GP64 Pseudotyped Bovine Immunodeficiency Virus-Based Lentiviral Vectors Efficiently Transduce Retinal Cells In Vivo*

Rene P. Molina, Hongqing Q. Ye, James Brady, Christine A. Mech, Michael Kaleko, Tianci Luo

**729**

*Receptor Trafficking Affects the Efficiency of Retrovirus Transduction*

Delfi Krishna, Joseph M. Le Doux

**730**

*Lentiviral Vectors Pseudotyped with Envelope Glycoproteins Derived from Human Parainfluenza Virus Type 3*

Cindy Jung, Bradley N. Grzybowski, Suxiang Tong, LiTeng Cheng, Richard W. Compans, Joseph M. Le Doux

**731**

*Pseudotyping of Lentiviral Vectors with New Envelope G Proteins from Vesiculoviruses VSV-New Jersey, Chandipura and Piry*

Akhil Banerjea, Ramesh Akkina

**732**

*A Novel Double-Pseudotyped Viral Vector for Gene Therapy*

Ning Chai, Gary Kobinger, Giancarlo Tanzi, James Wilson, Paul Bates

**733**

*Enhancement of Lentiviral-Mediated Gene Transfer in Kidney by Polycations and Detergents*

Elena V. Fedorova, Lorenzo Battini, Basil Hanss, G. Luca Gusella

**734**

*Efficiency of Preloading of Retroviral Vectors Without Fibronectin Peptide CH-296 Is Packing Cell Clone Dependent*

Claus S. Sondergaard, Christa Haldrup, Lene Pedersen

**735**

*EIAV-IGF-I Gene Transfer to Motor Neurons Enhances Axonal Length In Vitro*

Qinshan Teng, Mary Garrity-Moses, James K. Liu, Diana Tanase, Mimoun Azzouz, Lucy Walmsley, Nicholas D. Mazarakis, Nicholas M. Boulis

**736**

*Evaluation of an EIAV Gene Therapy Vector for Delivery of Therapeutic Antibodies*

Abigail Lamikanra, Kevin A. Myers, Kyri A. Mitrophanous, Susan M. Kingsman, Miles W. Carroll

**737**

*Development of a Feline Immunodeficiency Virus-Based Lentiviral Vector for Genetic Modification of CNS Neurons*

Geoffrey C. Owens, Craig T. Fredrickson, Edward W. Keefer

**738**

*Optimizing a Lentiviral Vector System for Efficient Transduction of Primary Rat Cardiovascular Cells In Vitro*

Jody R. Arsenault, Naohiro Hattan, William M. Chilian, Kurt J. Varner, Frank Park

**739**

*Lentiviruses as Gene Transfer Vehicles for Cancer Gene Therapy*

Riikka Pellinen, Tanja Hakkarainen, Tiina Pasanen, Kirsi Tulimaki, Anna Ketola, Anni Tenhunen, Pekka Timonen, Jarmo Wahlfors

**740**

*Replicating Retroviral Vector-Mediated Gene Transfer of the Chemokine, Interferon-Inducible Protein 10 (IP-10) Suppresses Angiogenesis and Tumor Growth in Mice*

Yuansheng Sun, Carsten Finger, Luis Alvarez-Vallina, Klaus Cichutek, Christian J. Buchholz

**741**

*VSV-G Pseudotyped, MLV-Based, Semi-Replication-Competent Retroviruses for Cancer Gene Therapy*

Jian Qiao, Jesus Moreno, Michael Forshaw, Rosa M. Diaz, Richard G. Vile

**742**

*Generation and Evaluation of a New Replication-Competent Retrovirus (RCR) Vector Coding for the SuperCD Suicide Gene*

Kai Hauschulz, Stefan Seefried, Andrea Schenk, Irina Smirnow, Wolfgang Wybranietz, Noriyuki Kasahara, Michael Bitzer, Ulrich M. Lauer

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Saturday, June 5

**743**

*Study of Lentiviral Vectors with Two Marker Genes Driven by Two Independent Promoters for Gene Transfer Applications*

Marilyne Dijon, Christian Chabannon, Caroline Torne-Celer

#### AAV Vectors: Genomes and Biology

**744**

*Genetic Structure of Adeno-Associated Virus DNA in Human Tissues*

Bruce C. Schnepf, Chun-Liang Chen, Ryan L. Jensen, K. Reed Clark, Philip R. Johnson

**745**

*Innate Immune Responses to Adeno-Associated Virus Vectors*

Anne K. Zaiss, Lindsay R. White, Norman C. W. Wong, Jeffrey S. Bartlett, Daniel A. Muruve

**746**

*Genomic Stability of Self-Complementary Recombinant Adeno-Associated Virus 2 in Mouse Muscle*

Changchun Ren, Sanjay Kumar, Selvarangan Ponnazhagan

**747**

*Direct Labeling and Tracking of Adeno-Associated Virus Single Stranded DNA in AAV Transduction*

Bernd Hauck, Wei Zhao, Weidong Xiao

**748**

*Generation of Novel Double-Stranded AAV Vectors with an Expanded Packaging Capacity Using Linearized 1-AAV ITR DNA Molecules*

Ognjen Petras, Timothy J. Davern, Ryan McTaggart

**749**

*Adeno-Associated Virus Vectors Integrate at Chromosome Breakage Sites*

Daniel G. Miller, Lisa M. Petek, David W. Russell

**750**

*AAV-2 Vectors and Protease-Activated Receptors: A Potential Interface between Coagulation and Gene Transfer*

Jianxiang Zou, Joerg Schuettrumpf, Alexander Schalchterman, Kian W. Tian, Jian-Hua Liu, Patricia Andrade-Gordon, Valder R. Arruda

**751**

*Breaking Down AAV: Defining the Minimal Capsid Sequences for AAV Function*

Yu-Hung Kuo, Jill M. Roberts, Michael G. Kaplitt

**752**

*Effect of 127 Mutations at 72 Positions on the Surface of Adeno-Associated Virus Type-2*

Michael Lochrie, Gwen Tatsuno, Jennifer Wellman, Shang-zhen Zhou, Richard Surosky, Glenn Pierce, Peter Colosi

**753**

*Evaluation of AAV Neutralizing Antibodies Using a Passive Immunity Model in SCID Mice*

Ciaran D. Scallan, Tongyao Liu, Susannah Patarroyo-White, Haiyan Jiang, Shangzhen Zhou, Glenn Pierce, Linda B. Couto

**754**

*Intracellular Trafficking Patterns of rAAV-2 Demonstrate Significant Cell-Type Specificity*

Wei Ding, Neal Zhang, Ziying Yan, Richard Peluso, Barrie Carter, John F. Engelhardt

**755**

*Cytoplasmic Dynein-Dependent Adeno-Associated Virus Interaction with Microtubules*

Samir Kelkar, Ronald G. Crystal, Philip L. Leopold

**756**

*Distinct Classes of Proteasome-Modulating Agents Cooperatively Augment Recombinant Adeno-Associated Virus Type 2 and Type 5-Mediated Transduction from the Apical Surface of Human Airway Epithelia*

Ziying Yan, Roman Zak, Yulong Zhang, Wei Ding, Simon Godwin, Keith Munson, Richard Peluso, John F. Engelhardt

**757**

*Enhancement of Gene Expression by a Recombinant Adeno-Associated Virus by Various Chemical Treatments*

Sung Jin Kim, Young Ran Nam, Chul-Hyen Joo, Jin Woo Chang, Yoo-Kyun Kim, Heuiran Lee

**758**

*A Histone Deacetylase Inhibitor Enhances Recombinant Adeno-Associated Virus-Mediated Gene Expression in Cancer Cells In Vitro and In Vivo*

Takashi Okada, Tatsuya Nomoto, Yuhe Liu, Mayumi Iwata-Okada, Masafumi Takahashi, Kuniko Shimazaki, Takashi Matsushita, Hiroaki Mizukami, Akihiro Kume, Keiya Ozawa

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Saturday, June 5

**759**

*The DNA Minor Groove Binding Agents Hoechst 33258 and 33342 Enhance Recombinant Adeno-Associated Virus (rAAV) Transgene Expression*

Lina Li, Linda Yang, Robert M. Kotin

**760**

*Dual Therapeutic Utility of Proteasome Modulating Agents for Pharmico-Gene Therapy of the Cystic Fibrosis Airway*

Liang N. Zhang, Phil Karp, Christopher J. Gerard, Eric Pastor, Keith Munson, Ziying Yan, Simon Godwin, Joseph Zabner, Richard Peluso, Barrie Carter, John F. Engelhardt

**761**

*Targeting DNA-PK by siRNA To Study AAV Replication*

Young-Kook Choi, Barry J. Byrne, Sihong Song

**762**

*Human Papillomavirus Type 16 Helper Functions for Adeno-Associated Virus Type 2 Replication*

Paul L. Hermonat, Yong Liu, Jawahar L. Mehta, Hong You

**763**

*Size-Dependent Packaging and Transgene Expression of Double-Stranded AAV Vectors*

Zhong Wang, Gregory Szeto, Jian Zhang, Juan Li, Xiao Xiao

**764**

*Role of Actin Cytoskeleton and Small GTPase Rap1 in the Co-Receptor Activity of Activated  $\alpha 5\beta 1$  Integrin for Human Parvovirus B19 Entry*

Kirsten A. Weigel-Kelley, Arun Srivastava

### Adenovirus Biology and Disease Applications

**765**

*The Efficacy of Adenovirus Vector-Mediated Gene Transfer into Human Osteoblasts In Vitro and Osseous Tissue In Vivo*

Keun Bae Lee, Charles L. Saltzman, Peter Taft, Michael Klotz, Joseph Zabner

**766**

*Human Hematopoietic (CD34+) Stem Cells Possess High Affinity Receptors for Adenovirus Type 11p*

Ya-Fang Mei, Anna Segerman, Kristina Lindman, Per Hörnsten, Anders Wahlin, Göran Wadell

**767**

*Intact Microtubules Are Required for Ad-Vector Induction of Chemokine Genes in Epithelial Cells*

Daniel A. Muruve, Lindsay R. White

**768**

*Activity of the Extracellular Domain of the Coxsackie and Adenovirus Receptor*

Katherine J. D. A. Excoffon, Geri L. Traver, Nicholas Gansemier, Joseph Zabner

**769**

*Microvessel Injury in Irradiation Damage in Salivary Glands*

Ana Paola Cotrim, Corinne M. Goldsmith, Anastasia L. Sowers, James B. Mitchell, Bruce J. Baum

**770**

*Pharmacogenetics of Adenoviruses*

Pierre Lefesvre, Dirk van Bekkum

**771**

*Adenovirus-Mediated siRNA Against Survivin Enhanced Sensitivity to Paclitaxel-Induced Apoptosis in MCF-7 Cells*

Toshihiro Tanaka, Hiroaki Uchida, Katsunori Sasaki, Makoto Miyagishi, Kazunari Taira, Hirofumi Hamada

**772**

*Adenoviral-Mediated Antioxidant Gene Transfer: A Gene Therapy Approach towards Diminishing Pancreatic  $\beta$ -Cell Susceptibility to Cytokine-Induced Oxidative Damage*

Cillian T. McCabe, Timothy O'Brien

**773**

*Non-Invasive In Vivo Detection of Episomes Delivered to Mouse Hepatocytes by a Helper Dependent Adenovirus- Epstein-Barr Virus Hybrid Vector System*

Sean D. Gallagher, Jose S. Gil, Oliver Dorigo, Arnold J. Berk

**774**

*Stability Study of an Adenoviral Vector Prepared in GTS: The Buffer Formulation of the Adenovirus Reference Material*

Ronald Gilbert, Francois Bouthillier, Bernard Massie

**775**

*An Novel Adenoviral E1 Complementary Cell for Eliminating Replication Competent Adenovirus (RCA) during Viral Production*

Deborah Farson, Lucy Tao, Derek Ko, Thomas Harding, De-Chao Yu, Yuanhao Li

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Saturday, June 5

**776**

*Adenoviral Mediated Gene Transfer of NOS Isoforms and Vascular Cell Proliferation*  
Ronan Cooney, Timothy O' Brien

**777**

*Inactivated Adenoviral Vectors with a Conditional Infectivity*  
Mark W. Pandori, Takeshi Sano

**778**

*Rapid Adenoviral Neutralization Assay*  
Edward C. Nwanegbo, Wentao Gao, Simon Barrett-Boyes, Paul D. Robbins, Andrea Gambotto

**779**

*High-Capacity Adenovirus/ Retrotransposon Hybrid Vectors for Efficient and Stable Gene Transfer*  
Shuji Kubo, Harris Soifer, John V. Moran, Haig H. Kazazian, Jr, Nori Kasahara

**780**

*Efficient Adenovirus-Mediated Human Interferon-gamma Transfer into C6 Glioma Cells*  
Lizhi Wu, Xiuju Wu, Wenlin Huang

**781**

*Quantification of Empty Capsids in Preparations of Conditionally Replicating Adenovirus*  
Sundari Ravindran, Xiaoyu Yang, Suganto Sutjipto, Yan-Hui Liu, Gary Vellekamp

**782**

*Genetic Labeling of Adenovirus with Red Fluorescent Proteins on pIX for Vector Detection*  
Long P. Le, Igor P. Dmitriev, Julia G. Davydova, Masato Yamamoto, David T. Curiel

**783**

*Compared Stability and Antitumour Activity of Recombinant Endostatin Protein Expressed in Yeast with Recombinant Adenovirus Carrying Endostatin Gene*  
Zihui Liang, Jialin Huang, wenlin Huang

**784**

*An AdEASY Based Cloning System for Generating Adenoviral Vectors That Express Transgenes Late in the Viral Life Cycle*  
Michael Lie-A-Ling, Conny Bakker, John G. Wesseling, Piter J. Bosma

**785**

*Synergism of Combined Radiation and Suicide Gene Therapy Using a Chimeric Adenovirus Vector for Bladder Cancer*

Kazumasa Matsumoto, Christian T. F. Freund, Weiguo Jian, Jiang Yu, Patricia Yotnda, Alan R. Davis, Seth P. Lerner

**786**

*Genetic Labeling of Adenovirus with Luciferase on pIX for Vector Detection*  
Long P. Le, Igor P. Dmitriev, Julia G. Davydova, Masato Yamamoto, David T. Curiel

**787**

*Cationic Lipids Improve Transfection Efficacy of Recombinant Replication Deficient Adenovirus in Cutaneous Cells*  
Frank Jacobsen, Tobias Hirsch, Dominik Mittler, Hans Ulrich Steinau, Lars Steinstraesser

**788**

*Imaging of Adenoviral Replication with a Fluorescent Reporter in the E3 Region*  
Hidetaka A. Ono, Long P. Le, Julia G. Davydova, Tatyana Gavrikova, Pedro J. Ramirez, Hiroshi Shimada, David T. Curiel, Masato Yamamoto

**789**

*Hydrodynamic Injection of Helper Dependent Adenoviral Vectors Increases Liver Transduction Efficiency and Decreases Acute Inflammatory Response*  
Nicola Brunetti-Pierri, Viraj Mane, Donna Palmer, Arthur L. Beaudet, Philip Ng

**790**

*Regulatable Transgene Expression from Gutless Adenoviral Vectors*  
Shyam Goverdhana, Weidong Xiong, Diana S. Greengold, James Curtin, Eric Soffer, Pedro R. Lowenstein, Maria G. Castro

**791**

*Histone Deacetylase Inhibition Up-Regulates CAR and Increases Adenovirus Infection and Transgene Expression in Skeletal Muscle*

Alan R. Brooks, Linda Cashion, Kaye Reiter, Peiyin Wang, Hu Sheng Qian, Richard N. Harkins, Gabor M. Rubanyi

**792**

*Dynamic High Capacity MUSTANG® Q Membrane Units for Scaleable Anion Exchange Chromatography Purification of Adenoviral Vectors*  
Robert H. Kutner, Ajay Lajmi, Jakob Reiser

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Saturday, June 5

**793**

*Adenopure LST™ Large Scale Kit for Purification of Recombinant Adenovirus*

Timothy D. Kierstead, Tawfiq Hazboun, Anthony P. Green

#### DNA Viruses: Other

**794**

*Immobilized Cobalt Affinity Chromatography Provides a Novel, Efficient Method for HSV Gene Vector Purification*

Canping Jiang, James B. Wechuck, William F. Goins, David M. Krisky, Darren P. Wolfe, Mohammad M. Ataai, Joseph C. Glorioso

**795**

*Highly Purified, In Vitro Constructed SV40 Vectors for In Vivo Gene Delivery*

Santanu Mukherjee, Michal Bronstein, Mahmoud Abd-El-Latif, Orly Ben-nun-Shaul, Ariella Oppenheim

**796**

*The HSV-1 iBAC Amplicon Vector for Functional Genomics Studies into the Genetic Mechanisms of Neurodegenerative Disease*

S. E. Lawler, S. Senior, M. Ingelsson, Y. Saeki, B. T. Hyman, A. P. Monaco, E. A. Chiocca, R. Wade-Martins

**797**

*A Trans-Dominant Negative and Replication Defective HSV-1 Vector for In Vivo and Ex Vivo Gene Transfer*

Christoph F. P. Theopold, Daniela Hoeller, Feng Yao

**798**

*Intracarotid Delivery of Oncolytic Herpes Simplex Virus-1 G47Δ to Metastatic Breast Cancer in the Brain*

Renbin Liu, Robert L. Martuza, Samuel D. Rabkin

**799**

*Initial Characterization of a Super-Permissive Cell Line for Adeno-Associated Virus (AAV): Microarray-Intimated CK2 Helps AAV Replication*

Hong You, Yong Liu, Jawahar L. Mehta, Paul L. Hermonat

**800**

*Generation of Recombinant Skin In Vitro by Adeno-Associated Virus Type 2 Vector Transduction*

Nalini Agrawal, Yong Liu, Jawahar L. Mehta, Hong You, Paul L. Hermonat

**801**

*Novel Targeting Non-AAV Parvoviral Vectors for Gene Therapy*

Refael Itah, Igor Etingov, Clay Davis, Jacov Tal

**802**

*Rapid Conversion of HSV Resistant Cells to Sensitive Cells Using Adv-Mediated Overexpression of HSV Receptors*

Shinji Yamamoto, Edyta Tyminski, E. A. Chiocca, Yoshinaga Saeki

**803**

*Functional Analysis of Various Gene Linkers Serving Proportional Coexpression of PET Marker and Therapeutic Genes*

Alexandra Winkeler, Markus Hartung, Mark Slack, Andreas H. Jacobs

**804**

*Expression of Green Fluorescent Protein Modifies the Antitumor Effect of Oncolytic HSV-1 Vectors*

Yasushi Ino, Yoshinaga Saeki, E. Antonio Chiocca, Tomoki Todo

**805**

*Efficient Transduction of Human Cord Blood and Adult Peripheral Blood CD34+ Progenitors with HSV-1 Derived Amplicon Vectors*

Martin Krahn, Anne-Marie Imbert, Catherine Badens, Cecile Zaupa, Alberto Epstein, Christian Chabannon, Michel Fontes

**806**

*HSV Based Genetic Selection System for Identification of Pain Signaling Regulators*

Rahul Srinivasan, Darren Wolfe, Shaohua Huang, David Krisky, Joseph Glorioso

**807**

*Hyper-Attenuated Vaccine Strain of Vaccinia Virus*

Bela Denes, Daila S. Gridley, Tatyana Timiryasova, Nadja Fodor, Zsuzsanna Takatsy, Istvan Fodor

#### Naked DNA-Based Gene Transfer II

**808**

*Electro-Tranfection of Epstein-Barr Virus LMP-2 Specific TCRs Provides a Non-Viral Means To Transfer Antigen Specific T-Cell Receptors to Activated PBMC Populations*

Lisa Jurgens, Jill Gershon, James Weber, Rimas Orentas

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Saturday, June 5

**809**

*PhiC31 Integrase Mediates Site-Specific Genomic Integration in Rabbit Cells and Rabbit Knee Joints*  
Annahita Keravala, Joan Nash, Joylette L. Portlock, Paul D. Robbins, Michele P. Calos

**810**

*Site-Specific Integration Enhances Expression of DNA Introduced into Skeletal Muscle*  
Thurman M. Wheeler, Sohail Jarrahan, Carmen Bertoni, Eric C. Olivares, Timothy Doyle, Christopher H. Contag, Michele P. Calos, Thomas A. Rando

**811**

*The Hotspots of Integration Targeted by the Site-Specific Integrase PhiC31 May Be Cell Line Dependent*  
Anja Ehrhardt, Jeffrey A. Engler, Michele P. Calos, Mark A. Kay

**812**

*Hydroporation as the Mechanism Accounting for Efficient Gene Transfer by Hydrodynamic Delivery*  
Dexi Liu, Guisheng Zhang, Xiang Gao, Young K. Song, Regis Vollmer, Donna B. Stoltz, Joshua Z. Gasiorowski, David A. Dean

**813**

*Ecdysone Receptor-Based Inducible Gene Regulation System for Simultaneous Regulation of Multiple Genes*  
Prasanna Kumar, Anand K. Katakam, Subba R. Palli, Mohan Kumar, Henry Hoff III, Dean E. Cress

**814**

*Integration Sites Used by the PhiC31 Site-Specific Integrase in the Human Genome*  
Thomas W. Chalberg, Daniel S. Ginsburg, Patrick J. Kirby, Eric C. Olivares, Joylette L. Portlock, Bhaskar Thyagarajan, Michele P. Calos

**815**

*Investigation into the Frequency of Post-Integrative Gene Silencing Using a Non-Selective Sleeping Beauty Transposition System*  
Brian S. Garrison, Stephen R. Yant, Jacob G. Mikkelsen, Mark A. Kay

**816**

*Development of Potent DNA Vaccines by Coupling of Linear MIDGE Vectors to Peptides*  
Sonia Moreno, Florian Sack, Colin Smith, Luisa Lopez-Fuertes, Antonio J. Vila-Coro, Sven A. König-Merediz, Burghardt Wittig, Matthias Schroff, Manuel Schmidt, Marcos Timon

**817**

*Nonrandom Insertion Site Preferences for the SB Transposon In Vitro and In Vivo*  
Stephen R. Yant, Xiaolin Wu, Yong Huang, Bernie Daigle, Brian A. Garrison, Shawn M. Burgess, Mark A. Kay

**818**

*Optimization of *E. coli* Fermentation for Plasmid DNA Production*  
Aaron Carnes, Clague Hodgson, Jim Williams

**819**

*Hyperactive Transposase Mutants of the Sleeping Beauty Transposon*  
Stephen R. Yant, Julie Park, Yong Huang, Jacob Giehm Mikkelsen, Mark A. Kay

**820**

*Cost-Effective, Pharmaceutical-Grade Plasmid Production Method for Commercial Use*  
Henry Hebel, Sriram Ramakrishnan, Hugo Gonzalez, Heather Attra

**821**

*A Potential Therapeutic Angiogenesis Using a Naked DNA Containing Angiogenin cDNA*  
Keerang Park, Soo-Ik Chang, Young-Il Lee, Heesoon Chang, Young-Hwa Cho, Bong-Su Kang, Jeong-Eun Lee, Ji-Eun Yun, Hyun Park

**822**

*Efficient Delivery of Plasmid DNA to Murine Skin Using In Vivo Electroporation*  
Richard Heller, Mark J. Jaroszeski, Loree C. Heller, Kathleen Merkler, Andrea N. McCray, Richard A. Gilbert

**823**

*Use of a Cre Recombinase Silencing Strategy to Characterize Sleeping Beauty-Mediated Transposition In Vivo*  
Paul R. Score, Joel L. Frandsen, Jennifer L. Geurts, Perry B. Hackett, David A. Largaespada, R.Scott McIvor

**824**

*Duration of Expression of Sleeping Beauty Transposase by Hydrodynamic Injection of C57/BL6 Mice*  
Jason B. Bell, Andrew C. Wilber, Elena L. Aronovich, John R. Ohlfest, Brenda L. Koniar, David A. Largaespada, R. Scott McIvor, Perry B. Hackett

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Saturday, June 5

**825**

*Downstream Processing of Supercoiled Plasmid DNA*  
Lena M. Sandberg, Annika Andersson, Jozsef Vasi,  
Marianne Sparrman, Emma Håkansson, Per Nilsson,  
Raf Lemmens

**826**

*Non-Viral Gene Therapy Targeting Fanconi Anemia Hematopoietic Stem Cells: A Realistic Hope?*  
Meenakshi Noll, Ryan Bennett, Stephen Yant, Jacob G. Mikkelsen, Cindy Chen, Mark A. Kay, Markus Grompe

**827**

*A Novel Bone Gene Therapy Platform Based on Nonvirally Transfected Human Mesenchymal Stem Cells*  
Hadi Aslan, Tamar Azoulay, Yoram Zilberman, Gadi Pelled, Jinzhong Li, Gregory A. Helm, Zulma Gazit, Dan Gazit

### Molecular Conjugates

**828**

*Evaluation of Non-Viral Vectors for Gene Therapy of Hemophilia A*  
Katherine Bowman, Rita Sarkar, Xuli Wang, Hai-Quan Mao, Kam W. Leong

**829**

*Preferential Transfection of Tissue Macrophages Following Intravenous Administration of sec-R Targeted hCFTR DNA Enhances Survival of CF Mice Inoculated with *Pseudomonas aeruginosa* Agar Beads*

Assem G. Ziady, James Poleman, Christiaan van Heeckeren, Anna van Heeckeren, Pamela B. Davis

**830**

*Efficient Transfer of Synthetic Small Interfering RNAs through Atelocollagen-Mediated Delivery System*  
Shunji Nagahara, Yoshiko Minakuchi, Fumitaka Takeshita, Kimi Honma, Koji Hanai, Akihiko Sano, Takahiro Ochiya

**831**

*Biodistribution of DNA-Complex of Dendritic Poly (L-Lysine) after Intravenous Injection*  
Takahito Kawano, Tatsuya Okuda, Takuro Niidome

**832**

*Radioscintigraphy of sec-R Directed CFTR Gene Transfer in CF Mice Following Intranasal Administration*  
Assem G. Ziady, Gregory Wojtkiewicz, Pamela B. Davis, Zhenghong Lee

**833**

*Metabolically Stabilized Non-Viral Gene Delivery Systems*  
Dijie Liu, Molly E. Martin, Changpo Chen, Kevin G. Rice

**834**

*Tumor Targeted Gene Delivery Using a Non-Viral Vector System*  
Puthupparampil V. Scaria, Raymond M. Schiffelers, Aslam Ansari, Gert Storm, Qin Zhou, Martin Woodle

**835**

*Macropinocytosis of Polyplexes and Recycling of Plasmid from Clathrin-Dependent Pathway Impair the Transfection Efficiency into Human Hepatocarcinoma Cells*  
Christine Gonçalves, Eric Mennesson, Renate Fuchs, Jean-Pierre Gorvel, Patrick Midoux, Chantal Pichon

**836**

*Transendothelial Transport Studies of Polymer-Based Gene Delivery Systems*  
Eric Mennesson, Pierre Sandrin, Claudine Kieda, Patrick Erbacher, Patrick Midoux, Chantal Pichon

**837**

*Polyethylenimine/Albumin Complexes Display High Transfection Efficiency in Confluent and Polarized Human Respiratory Epithelial Cells*  
Salvatore Carrabino, Massimo Conese

**838**

*PEI-Chitosan Graft Copolymers for Mucosal Delivery of Genetic Vaccines*  
Sudhir P. Kasturi, Krishnendu Roy

**839**

*The Effect of PEGylation on Polyplexes Containing Ligands: Morphology and Binding Efficiency*  
Yan Guo, Jianren Gu, Yuhong Xu

**840**

*Novel Receptor-Mediated Gene Transfection Systems Comprising Plasmid / Polycation / RGD-PEG-COOH Ternary Complexes*  
Mitsuko Sakae, Tomoko Ito, Naoko Iida Tanaka, Takuro Niidome, Hironobu Yanagie, Yoshiyuki Koyama

**841**

*Cysteine-Flanked Melittin Analogues as Lysotropic Gene Delivery Vectors*  
Chang-Po Chen, Ji-seon Kim, Kevin G. Rice

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Saturday, June 5

**842**

*Cell Permeable Peptides: Potential Use To Enhance Intracellular Gene Delivery*

Miguel Mano, Paula Verissimo, Carlos Faro, Abraham Loyter, Sergio Simoes, Maria C. Pedrosa de Lima

**843**

*Single-Chain Polypeptide: Universal Module for Targeted Gene Delivery*

Valentina A. Anisimova, Igor G. Shemyakin

**844**

*Novel Pathways of Cell Entry and Trafficking Utilized by Ad Capsid Proteins in the Absence of the Whole Virus*

Altan Rentsendorj, Hasmik Agadjanian, Maguire Meghan, Jiansong Xie, Xinhua Chen, Michelle MacVeigh, Hamm-Alvarez Sarah, Lali K. Medina-Kauwe

**845**

*Enhanced Efficacy of Radiation Sensitivity by Controlled Gene Delivery of PTEN Expression Vector Conjugated with Cationized Gelatin in Prostate Cancer Cells*

Yoshiaki Matsumura, Satoshi Takada, Satoshi Anai, Motoyoshi Tanaka, Hirotugu Uemura, Yoshihiko Hirao

**846**

*Copolymer-Protected Vector Mediated BMP-2 Gene Delivery to Articular Chondrocytes in a 3-Dimensional Injectable Fibrin Matrix for Tissue Engineering*

Ulrike Schillinger, Gabi Wexel, Christian Hacker, Martin Kullmer, Axel Stemberger, Christian Plank

**847**

*Hyaluronic Acid as a Self-Assembled Coating of Plasmid/Polycation Complexes for Cell-Specific Gene Delivery*

Tomoko Ito, Naoko Iida Tanaka, Toshinori Sato, Zhihong Yang, Takuro Niidome, Takahito Kawano, Yoshiyuki Koyama

**848**

*The Study on the VEGF Plasmids Purify by Magnetic Nanoparticles*

Minli Sun, Hao Zhang, Baigen Zhang

**849**

*CD Spectra of Compacted DNA Nanoparticles*

*Demonstrate a Unique DNA Structure*

Tomasz H. Kowalczyk, Christopher R. Gedeon, Patrick J. Klepcyk, Sharon M. Oette, Tamara L. Fink, Robert C. Moen, Mark J. Cooper

**850**

*Quantitative Analysis of Non-Viral Gene Therapy in a Three-Dimensional Liver Tissue Construct*

Nathan C. Tedford, David W. Jackson, Karel Domansky, Linda G. Griffith, Douglas A. Lauffenburger

**851**

*Development of Brain Tumor Organ Cultures for Characterizing Polymer/Plasmid DNA Mediated Gene Transfer*

Nathalie Y. Toussaint, Sean Kearns, Bjorn Scheffler, Ronald Mandel, Sean M. Sullivan

**852**

*Development of Systemic Non-Viral Gene Delivery Systems: Cationic Nanogels*

Serguei V. Vinogradov, Alexander V. Kabanov

### Inborn Errors of Metabolism: Lysosomal Storage and Other Diseases

**853**

*Efficacy of AAV-Mediated Expression of Acid Sphingomyelinase at Correcting the Visceral and Pulmonary Manifestations of Niemann-Pick B Disease*

C. M. Barbon, R. J. Ziegler, S. D. Bercury, M. Cherry, C. Li, E. Schuchman, R. J. Desnick, M. J. Lukason, S. Lonning, S. H. Cheng

**854**

*Feasibility of AAV-Mediated Gene Therapy Examined Using a New Murine Model (D409V/null) of Gaucher Disease*

John Marshall, Kerry McEachern, Jennifer B. Nietupski, Julie A. Cavanagh Kyros, Donna Armentano, Gregory A. Grabowski, Seng H. Cheng

**855**

*Stable Transduction of Hepatocytes Following Intravenous Retroviral Gene Therapy in Seven-Week-Old Dogs with Mucopolysaccharidosis (MPS) VII*

Mark Haskins, Thomas O'Malley, N. Matthew Ellinwood, Patricia O'Donnell, Kristen Cullen, Karyn Cullen, Margaret Sleeper, Gustavo Aguirre, Lingfei Xu, Ping Wang, Katherine Ponder

**856**

*Neonatal Liver-Directed Gene Therapy Markedly Reduces Bone Disease in MPS I*

Yuli Liu, Mango Robert, Lingfei Xu, Deborah Novack, Clay Semenkovich, Trey Coleman, Katherine Ponder

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Saturday, June 5

**857**

*Intravenous Retroviral Gene Therapy in Neonatal Mucopolysaccharidosis VI Cats*  
Thomas M. O'Malley, YuLi Liu, Patty A. O'Donnell, Karyn J. Cullen, Ping Wang, Kristen J. Cullen, Gustavo D. Aguirre, Katherine P. Ponder, Mark E. Haskins

**858**

*Correction of the CNS Pathology and Function of MPS VII Mice by Intraventricular Transplantation of Genetically Modified Bone Marrow Stromal Cells*  
Ken Sakurai, Sayoko Iizuka, Jin Song Shen, Xing Li Meng, Taisuke Mori, Akihiro Umezawa, Toya Ohashi, Yoshikatsu Eto

**859**

*A Skin-Restricted Conditionally Expressing Mouse Model of Pompe Disease for Assessment of Gene and Enzyme Replacement Therapies*  
Kerry O. Cresawn, Nina Raben, Paul H. Plotz, Christina Pacak, Adam Glick, Barry J. Byrne

**860**

*Long-Term Correction of Lysosomal Storage in CNS and Somatic Tissue in Adult MPS II Mice by Intravenous Administration of AAV*  
Joseph Muenzer, Jerillyn S. Jennings, Haiyan Fu

**861**

*Neonatal Gene Therapy with a Lentiviral Vector Results in Therapeutic Levels of  $\alpha$ -Galactosidase A Correction in Fabry Mice*  
Makoto Yoshimitsu, Kesheng Tao, Xiaohu Fan, Lori West, Jeffrey A. Medin

**862**

*Improved Efficacy of Adenovirus-Mediated Gene Therapy for GSD-II Disease by Selective Depletion of Kupffer Cells*  
Shaoxi Liao, Ruth Everett, Fang Xu, Delila Serra, Nico Van Rooijen, Andrea Amalfitano

**863**

*Expression and Secretion of Human Glucocerebrosidase Mediated by Recombinant Lentivirus Vectors In Vitro and In Vivo*  
Eun Young Kim, Young Bin Hong, Zhennan Lai, Roscoe O. Brady, Sung-Chul Jung

**864**

*AAV-2-Mediated Expression of  $\alpha$ -N-Acetyl-Glucosaminidase in Peripheral Tissues and the CNS in Mucopolysaccharidosis IIIB Mice by Combined Intravenous and Intracisternal Vector Delivery*  
Lu Kang, Jerillyn S. Jennings, Joseph Muenzer, Haiyan Fu

**865**

*An Ex Vivo Gene Therapy Approach for Metachromatic Leukodystrophy Using Neural Progenitor Cells*  
Ken Kawabata, Makoto Migita, Hideki Mochizuki, Yoshitaka Fukunaga, Takashi Shimada

**866**

*Modification of the Expression Vector for Systemic Gene Therapy of MLD*  
Yukiko Takakusaki, Sanae Hisayasu, Atsushi Watanabe, Takashi Shimada

**867**

*Correction of Glycogen Storage Disease Type II by Intramuscular Administration of an Adeno-Associated Virus 6 (AAV2/6) Vector Containing a Muscle-Specific Promoter*  
Haoyue Zhang, Baodong Sun, Luis Franco, Andrew Bird, Ayn Schneider, Dwight D. Koeberl

**868**

*Enhancement of Gene Therapy Approaches for the Correction of Farber Disease*  
Shobha M. Ramsubir, Claudine Tardy, Stephane Carpentier, Thierry Levade, Jeffrey A. Medin

**869**

*Unique Potential of Gene Therapy for GSD-II Using Fully Deleted Adenovirus Based Vectors Expressing hGAA*  
Anne Kiang, Fang Xu, Shiao Xi Liao, Donna J. Palmer, Philip Ng, Andrea Amalfitano

**870**

*Gene Transfer in the Sandhoff Murine Model Using Lentiviral Vectors*  
Audrey Arfi, Christophe Bourgois, Luisa Basso, Sandro Sonnino, Livia Poenaru, Catherine Caillaud

**871**

*Long-Term Expression of Sleeping Beauty Transposon in the Murine Model of Mucopolysaccharidosis (MPS) Type VII*  
Elena L. Aronovich, Jason B. Bell, Lalitha R. Belur, Joel L. Fransden, Roland Gunther, Brenda Koniar, David C. C. Erickson, R. Scott McIvor, Perry B. Hackett, Chester B. Whitley

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

**872**

*AAV 1 and 8 Have Higher Efficacy Than AAV 2 in Correction of Hyperbilirubinemia in the Gunn Rat, the Animal Model for the Metabolic Liver Disorder Crigler-Najjar Syndrome*

Piter J. Bosma, Berry de Jong, Norbert Looije, Cindy Kunne, Conny T. Bakker, Jurgen Seppen

**873**

*Low Level Hepatocyte Repopulation Corrects Hyperphenylalaninemia in Murine Phenylketonuria*

Cary O. Harding, Kelly Hamman, Heather Clark, Eugenio Montini, Muhsen Al-Dhalimy, Markus Grompe, Milton Finegold

**874**

*Hepatocellular Morphology in the Pahenu2 Mouse Model of PKU Is Altered Following Portal Vein Administration of rAAV-mPAH-WPRE Vectors*

Jeniffer E. Embury, Catherine E. Charron, Philip J. Laipis

**875**

*Ribozyme Approaches towards Down-Regulation of Pi<sup>Z</sup> Mutant Human a-1 Anti-Trypsin*

Thomas J. Conlon, Stuart G. Beattie, Chris W. Snyder, Alfred S. Lewin, Terry R. Flotte

**876**

*Towards Establishing a Heterologous Liver in Rodents*

Kazuo Ohashi, Takashi Yokoyama, Hiroyuki Kuge, Yoshiyuki Nakajima, Mark A. Kay

**877**

*Evidence for Dominant-Negative Interference in the Pahenu2 Mouse Model of PKU*

Catherine E. Charron, Alfred S. Lewin, Philip J. Laipis

**878**

*Correction of Maternal Phenylketonuria Syndrome in the Pahenu2 Missense Mutant Mouse by r-AAV Mediated Gene Therapy*

Philip J. Laipis, Catherine E. Charron, Jennifer E. Embury, O. P. Perera, Stacey L. Porvasnik, Carl R. Fields, Roberto T. Zori

**879**

*Morphologic and Immunohistochemical Evaluation of Dopaminergic Regions of Pahenu2 Mouse Brains*

Following Adminstration of rAAV-mPAH-WPRE Vector Jennifer E. Embury, Catherine E. Charron, Philip J. Laipis

Saturday, June 5

### Bone and Cartilage

**880**

*Suppression of Inflammation in a Rat Model of Arthritis Following Intramuscular Administration of AAV-TNFR:Fc Vector Pseudotyped with AAV Type 1 Capsid*

Ziv Sandalon, Kurt Lustig, Haim Burstein

**881**

*Preclinical Safety Assessment of Intra-Articular Administration of rAAV-humanTNFR:Fc in Rats in Support of a Phase I Arthritis Trial*

Laurie M. Tatalick, Linda D. Wilson, Tara L. Allen, Dana B. Martin, Pervin Anklesaria

**882**

*Adenovirus-Mediated Gene Transfer of Human Growth and Differentiation Factor-5 (GDF-5) into the Healing Rat Achilles Tendon*

Haili Wang, Markus Rickert, Peter Wieloch, Helga Lorenz, Eric Steck, Wiltrud Richter

**883**

*Non Viral Gene Therapy by Electrotransfer of hTNF- $\alpha$  Soluble Receptor-I Variants and Its Application to the Treatment of Experimental Arthritis*

Carole Bloquel, Natacha Bessis, Christophe Boissier, Daniel Scherman, Pascal Bigey

**884**

*Gene Gun Particle Encoding Preproenkephalin (PPE) cDNA Produces Analgesia Against Capsaicin Induced Bladder Pain in Rats*

Yao-Chi Chuang, Hong-Yo Kang, Po-Hui Chiang, Michael B. Chancellor, Naoki Yoshimura

**885**

*VEGF Enhances Bone Formation and Bone Healing Elicited by Transduced Muscle-Derived Stem Cells Expressing Human BMP2*

Hairong Peng, Arvydas Usas, Brian Gearhart, Johnny Huard

**886**

*Determination of Osteoprogenitor-Specific Promoter Activity in Murine Mesenchymal Stem Cells by Recombinant Adeno-Associated Virus Transduction*

Sanjay Kumar, Gandham Mahendra, Selvarangan Ponnazhagan

**887**

*Bone Formation by Ex Vivo Gene Therapy Using Lentiviral Vector Expressing BMP-2 under the Control of the MLV Promoter*

Osamu Sugiyama, Dong Sung An, Seth Gamradt, Nancy Q. Liu, Irvin S. Y. Chen, Jay R. Lieberman

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Saturday, June 5

**888**

*A BMP2/7 Fusion Gene Enhances Osteoblastic Differentiation in Mesenchymal Cells*

Wei Zhu, Oheneba Boachie-Adjei, Bernard Rawlins, Ronald G. Crystal, Chisa Hidaka

**889**

*Relationship between the Amount of Bone Formation and Protein Expression in Two Rat Strains Using Direct Adenoviral Vector Injection and Ex Vivo Method*  
Jin Zhong Li, Hongwei Li, El Noh, Lindsey Jacobson, An-Hsiang Liu, Dwight Saulle, Gerald R. Hankins, Gregory A. Helm

**890**

*Comparison of the Osteogenic Potential of AAVhBMP6 and ADhBMP6 between Two Rat Strains*  
Jin Zhong Li, Hongwei Li, An-Hsiang Liu, Lindsey Jacobson, El Noh, Dwight Saulle, Gerald R. Hankins, John A. Chriorini, Gregory A. Helm

**891**

*Effect of the Timing of Direct Adenoviral Gene Delivery on the Healing of Critical Sized Bone Defects in Rats*

Oliver B. Betz, Marcus Egermann, Volker M. Betz, Ara Nazarian, Andre F. Steinert, Glyn D. Palmer, Mary L. Bouxsein, Mark S. Vrhas, Thomas A. Einhorn, Christopher H. Evans

**892**

*Enhanced Survival of Chondrocytes Over Expressing Insulin-Like Growth Factor I in Equine Cartilage Repair*  
Laurie R. Goodrich, Chisa Hidaka, Lisa M. Strassheim, Paul D. Robins, Evans H. Chris, Alan J. Nixon

**893**

*Effect of Hepatic Insulin Gene Therapy on Intrahepatic Carbohydrate Metabolism In Vivo*  
Darin E. Olson, Adam G. Campbell, Peter M. Thule'

**894**

*Targeting Adenovirus Mediated Gene Transfer to Chondrocytes In Vitro*

Michael P. Seiler, Margaretha Guenther, Skip Mercier, Donna Palmer, Phillip Ng, Charlott Brunmark, Michael A. Barry, Brendan H. Lee

**895**

*The Differential Effect of BMP4 on C2C12 and NIH/3T3 Cells: Implications for Gene Therapy for Bone and Cartilage Repair*

Guangheng Li, Hairong Peng, Karin Corsi, Arvydas Usas, Johnny Huard

**896**

*Local Direct Injection of BMP2-Modified Fibroblasts Induces Ectopic Bone Formation in Immunocompetent Mice*

Enrico Pola, Wanda Lattanzi, Giovanni Pecorini, Pierangelo Papaleo, Giuseppe Straface, Carlo A. Logroscino, Andrea Gambotto, Paul D. Robbins

### Gene Transfer Applications for Disorders of Hematopoietic Cells

**897**

*The Development of Standards for Cell and Gene Therapy Products: The Activities of the United States Pharmacopeia*

Flavia Borellini, Sally S. Seaver, Ian F. DeVeau, Beth M. Hutchins

**898**

*Human Hematopoietic Repopulating Stem and Progenitor Cells Can Be Efficiently Selected Based on High Aldehyde Dehydrogenase Activity*

David A. Hess, Todd E. Meyerrose, Louisa Wirthlin, Phillip E. Herrbrich, Timothy P. Craft, Jan A. Nolta

**899**

*Stable Gene Expression under the Control of the  $\beta$ -Globin LCR in Hematopoietic Cells after Transduction with Integrating Helper-Dependent Adeno-AAV Vectors*

Hongjie Wang, Dmitry Shayakhmetov, Qiliang Li, Thalia Papayannopoulou, George Stamatoyannopoulos, André Lieber

**900**

*Development of Models for RPS19 Deficient Diamond-Blackfan Anemia Using Regulatable Expression of siRNA Against RPS19*

Koichi Miyake, Johan Flygare, Thomas Kiefer, Johan Richter, Isao Hamaguchi, Maciej Wiznerowicz, Didier Trono, Stefan Karlsson

**901**

*Transactivation of Fetal Hemoglobin Genes by Engineered Zinc-Finger Protein Transcription Factors for the Treatment of Sickle Cell Disease*

Andreas Reik, Jeffrey D. Milner, Tom Ryan, Philip D. Gregory, Tim Townes, Casey C. Case

**902**

*A Novel In Vivo, Lentivirus-Based Model of  $\alpha$ -Spectrin Function*

Sara Mootien, Nancy E. Siedel, David M. Bodine, Patrick G. Gallagher

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Saturday, June 5

**903**

*Group I Ribozymes and SMaRT™ as Trans-Splicing RNA Repair Therapies for β-Globin Mutations*  
Monique N. Kierlin, Jonghoe Byun, Ning Lan, Meredith Long, Bruce Sullenger

**904**

*Lentiviral Transduction of Wiskott-Aldrich Syndrome (WAS) Deficient, T-Cells Leads to Long-Term and Progressive WAS Protein Expression and Functional Reconstitution*

Carol H. Miao, Qili Zhu, Ling Zhou, Stephanie Humboldt-Baron, Datien Lin, Donald B. Kohn, Fabio Candotti, Hans D. Ochs, David J. Rawlings

**905**

*The Endogenous WASP Promoter Fragments in SIN Lentiviral Vectors Drive Expression Preferentially in T Cells*

Xiangyang Jin, Denise A. Carbonaro, Dinithi Senadherra, Denise Petersen, Sau-Ping Kwan, Donald B. Kohn

**906**

*Successful Functional Correction of T Cells from Wiskott-Aldrich Syndrome Patients with Lentiviral Vectors*

Loïc Dupré, Francesco Marangoni, Antonia Follenzi, Sara Trifari, Silvana Martino, Shigeru Tsuchiya, Claudio Bordignon, Luigi Naldini, Alessandro Aiuti, Maria Grazia Roncarolo

**907**

*T Lymphocyte-Directed Gene Therapy for IL-12R $\beta$ 1 Deficiency*

Marita Bosticardo, Claire Fieschi, Francesco Novelli, Jean-Laurent Casanova, Fabio Candotti

**908**

*Selective Enrichment of Transduced Cells for Gene Therapy of an Inherited Immune Deficiency, Chronic Granulomatous Disease*

Olga Ujhelyi, Judit Cervenak, Csilla Laczka Ozvegy, Manuel Grez, Balazs Sarkadi, Katalin Nemet

**909**

*In Vivo Repopulating Ability of Genetically-Corrected BM Cells from Fanconi's Anemia A Patients*  
Odile Y. Cohen-Haguenauer, Christian Auclair, Michel Marty

**910**

*Hematopoietic Cell Differentiation of Common Marmoset (*Callithrix jacchus*) Embryonic Stem Cells and Their Genetic Manipulation Using the Third Generation Lentiviral Vector*

Ryo Kurita, Erika Sasaki, Takashi Hiroyama, Yukoh Nakazaki, Kiyoko Izawa, Hajime Ishii, Yoshikuni Tanioka, Kisaburo Hanazawa, Makoto Osonoi, Takao Hashiguchi, Yuan-Son Bai, Yasushi Soda, Xiao jin Li, Sumiko Watanabe, Shigetaka Asano, Kenzaburo Tani

**911**

*MHC Class I and II Mismatched Marrow Stromal Cells from C57Bl/6 Mice Are Immune Rejected by Recipient Balb/c Mice*

Nicoletta Eliopoulos, John Stagg, Laurence Lejeune, Jacques Galipeau

**912**

*Strategies Using Retroviral Coexpression of Indoleamine 2,3 Dioxygenase and Fas Ligand for Allogeneic Bone Marrow Transplantation*

Bagirath Gangadharan, Lucienne Ide, David Holtzclaw, David Archer, H. Trent Spencer

**913**

*MGMT (P140K) Allows for Safe, Efficient, and Sustained In Vivo Selection and Chemoprotection in a Large Animal Model*

Brian C. Beard, Tobias Neff, Laura J. Peterson, Bobbie Thomasson, Jesse Thompson, Hans-Peter Kiem

**914**

*Mutant MGMT Lentivirus Co-Transduction with a Marker Lentivirus Efficiently Enriches for Dual-Vector Expressing Cells In Vivo*

Justin C. Roth, Mourad Ismail, Giuliana Ferrari, Stanton L. Gerson

**915**

*Insertional Analyses in Rhesus Monkey Blood Cells after Non-Myeloablative Hematopoietic Stem Cell Marking with a Therapeutic Onco-Retroviral Vector for X-Linked Chronic Granulomatous Disease*

Martin F. Ryser, Sebastian Brenner, Narda Whiting-Theobald, Romy Lehmann, Uimook Choi, Gilda F. Linton, Elizabeth Kang, Andrew G. Rudikoff, Ann M. Farese, Thomas J. MacVittie, Mitchell E. Horwitz, Harry L. Malech

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

**916**

*Systematic Analysis of Retroviral Vector Integrations in Human Marrow-Repopulating Cells by the Aid of a Data Warehouse*

Stephanie Laufs, K. Zsuzsanna Nagy, Guillermo Guenechea, Frank A. Giordano, Africa Gonzalez-Murillo, Kurt Fellenberg, Agnes Hotz-Wagenblatt, Juan A. Bueren, W. Jens Zeller, Stefan Fruehauf

**917**

*Long-Term Transgene Expression Following Gene Delivery Directly to the Bone Marrow Using Recombinant SV40-Derived Vectors*

Bianling Liu, Judy Daviau, David S. Strayer

**918**

*Autologous Canine Bone Marrow Stromal Cells Retrovirally Engineered with the Canine EPO Gene and Implanted In Vivo in a Collagen Matrix Produce and Release Systemically Functional EPO over a Prolonged Period*

Francois Fontaine, Marilyn Dunn, Juan Hernandez, Nicoletta Eliopoulos, Helene Boucher, James N. MacLeod, Jacques Galipeau, Daniel Martineau

**919**

*Gene Therapy for Platelet Disorders: Targeting Transgene Expression in Canine Platelets*

Lily M. Du, David A. Wilcox

**920**

*Assessing the Risk from Transfer of an Integrin  $\beta 3$  Gene That Encodes a Naturally Occurring Polymorphism Associated with Acute Coronary Thrombosis*

Juan Fang, Bryon D. Johnson, David A. Wilcox

**921**

*Biopump: A Novel Approach to Gene-Mediated Protein Production and Delivery with Application for Erythropoietin Treatment of Anemia*

Einat Brill-Almon, Andrew Pearlman, Baruch Stern, Noga Langer, Daniel Afik, Menachem Shavit, Yitzhak Lippin, Amos Panet, Ethan Galun, Eduardo Mitrani, Noam Shani

**922**

*Deriving Tissues from Embryonic Stem Cells*

Gordon Keller, Atsushi Kubo, Hans Joerg Fehling, Valerie Kouskoff

**923**

*Modeling Hematopoietic Transplantation with ES Cells*

George Q. Daley

**Saturday, June 5**

### Cardiac Gene and Cell Therapy

**924**

*Gene Therapy with IGF-1 Growth Factor for Heart Failure*

Armelle Serose, Andre Salmon, Bernard Prudhon, Marie Agnes Doyennette, Marc Y. Fiszman, Yves Y. Fromes

**925**

*Electroporation Mediated Gene Transfer of Human Interleukin-10 to Skeletal Muscle Reduces Acute Rejection in Rat Cardiac Allografts*

Reza Tavakoli, Amiq Gazdhar, Jaroslaw Pierog, Anna Bagdanova, Mathias Gugger, Steven Hyde, Michele Genoni, Ralph Schmid

**926**

*Bcl-xL Gene Transfer Improves Graft Survival and Prolongs the Cold-Preservation Time in the Heart Transplantation*

Jinghua Huang, Yoshinori Ito, Takeshi Uzuka, Masayoshi Kobune, Yukiko Honma, Kiminori Nakamura, Hiroaki Uchida, Hironari Dehari, Hirofumi Hamada

**927**

*Cell Therapy To Restore Perfusion and Cardiac Function in Infarcted Heart Is Improved by EPAS1 Gene Transfer*

Louis-Georges Guy, Yoshihisa Morimoto, Sandra Iacampo, Anouk S. Fortin, Bryce H. Davis, Guy Leclerc, Doris A. Taylor

**928**

*Hypoxia-Regulated Heme Oxygenase-1 Vector Confers Cardioprotection Against Ischemic Damage*

Yao Liang Tang, Yi Tang, Y. Clare Zhang, Keping Qian, M. Ian Phillips

**929**

*Target Adult Stem Cells to Ischemic Hearts by Regulated Stromal Cell-Derived Factor 1 Gene Transfer*

Yao Liang Tang, Keping Qian, Leping Shen, M. Ian Phillips, Y. Clare Zhang

**930**

*Muscle Stem Cells Deliver Dystrophin and Adopt a Cardiac Phenotype through Both Differentiation and Fusion in the Dystrophic ( $mdx$ ) Heart*

Thomas R. Payne, Hideki Oshima, Tetsuro Sakai, Yiqun Ling, Burhan Gharabeih, James H. Cummins, Johnny Huard

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Saturday, June 5

**931**

*Enhanced Cardiac Muscle Contractility Achieved by Engineered Zinc Finger Transcriptional Repressors of Phospholamban*

H. Steven Zhang, Lei Zhang, Yuxin Liang, Reed Hickey, Dmitry Guschin, Simon Chandler, Mike Kunis, Linda Hin, Dengfeng Xia, Xiaohong Zhong, S. Kaye Spratt, J. Tyler Martin, Casey C. Case, Frank J. Giordano, Philip D. Gregory, Edward J. Rebar

**932**

*SV40 Pseudovirions Are Efficient and Safe Myocardial Gene Therapy Vectors*

Ronen Beeri, Thea Pugatsch, Suzane Abedat, Mahmud Abdel Latif, Ariella Oppenheim

**933**

*Sendai Virus Vector Can Efficiently Introduce Transgene into Cardiomyocytes In Vitro and In Vivo*  
Yukiko Honma, Yoshinori Ito, Hironari Dehari, Masayoshi Kobune, Jinghua Huang, Kiminori Nakamura, Takeshi Kobayashi, Takeshi Uzuka, Sachie Hirai, Hiroaki Uchida, Makoto Inoue, Mamoru Hasegawa, Masayuki Morikawa, Noritsugu Tohse, Tomio Abe, Hirofumi Hamada

**934**

*Comparing Two Modalities of Myocardial Gene Delivery: Percutaneous Retrograde Coronary Venous Delivery and Intramyocardial Injection*  
Eyas Al-Shaykh Youssef, Ping Zhang, Pamela Rogers, Patrice Tremble, Joe Rokovich, Brian Johnstone, Keith L. March, Dongming Hou

**935**

*Magnetofection and Ultrasound To Increase "Naked" DNA Delivery to the Myocardium*  
Uta Griesenbach, Philip Dean, Nicola Marshall, Hai-Dong Liang, Stefania Xenariou, Duncan M. Geddes, Martin J. Blomley, Christian Plank, Eric W. F. W. Alton

**936**

*Phage-Selected Motifs Internalize Via Multiple Endocytic Pathways and Facilitate Gene Delivery*  
Zhengrong Hao, Yan Huang, Dinggang Liu, Jun Yu, Bill Sessa, Frank J. Giordano

**937**

*siRNA Inhibition of MMP9 Expression Inhibits TGF $\beta$ 1-Induced Calcification of Aortic Valve Interstitial Cells*  
Jocelyn N. Clark, Elizabeth Palmatory, Robin Brittin, Navneet R. Narula, Joe Gorman, Robert C. Gorman, Robert J. Levy

**938**

*Myocardial Administration of Adenoviral Vector Encoding VEGF Induces Severe Pleural and Pericardial Effusion in Rat*

Yoshinori Ito, Masayoshi Kobune, Takeshi Uzuka, Jinghua Huang, Yukiko Honma, Hironari Dehari, Hikaru Ueno, Hirofumi Hamada

**939**

*Adenoviral-Mediated Neuronal-Specific Overexpression of the Angiotensin AT2 Receptor*  
Hongwei Li, Yongxin Gao, Mohan K. Raizada, Colin Sumners

**940**

*Ribozyme Oligonucleotides Against TGF- $\beta$  Transferred by Ultrasound Inhibited the Progression of Diseases in Anti-Thy1 Model*

Naruya Tomita, Keita Yamasaki, Kei Yamamoto, Hiromi Koike, Yasuo Kunugiza, Toshio Ogihara, Ryuichi Morishita

### Cancer Tumor Suppressor Gene and Apoptosis

**941**

*Development of Cell Penetration Domain and Cell Killing Domain Genes for Treatment of Cancer*  
Sean M. Sullivan, Harm J. Knot, Ron Mandel, J. P. Jarajapu, Jacqueline Baltunis

**942**

*Combinatorial Treatment with Tumor Necrosis Factor-Related Apoptosis-Inducing Ligand (TRAIL) Gene and a Chemotherapeutic Agent Suppressed Hepatic Metastasis of Colon Carcinoma*

Michiaki Ishii, Masaki Iwai, Yoshinori Harada, Jiro Imanishi, Osamu Mazda, Takeshi Okanoue

**943**

*Loss of Neoplastic Phenotype by Specific Inhibition of RET Oncogene Signalling in a Transgenic Model of Primary Medullary Thyroid Carcinoma*  
Matthias Drosten, Gero Hilken, Florian Rodicker, Miriam Bockmann, Carmen C. Theseling, Nikica Mise, Aaron N. Cranston, Andreja Frilling, Bruce A. Ponder, Brigitte M. Putzer

**944**

*The Histone Deacetylase Inhibitor Depsipeptide (FR901228) Enhances the Cytotoxic Activity of Ad5-TRAIL Against Human Prostate Tumor Cells*  
Thomas S. Griffith, Jill Moore, Rebecca Van Oosten

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Saturday, June 5

**945**

*E1B-Mutant Replication Competent Adenovirus Combined with Chemotherapeutic Agents Shows Increased Anti-Tumor Effect*  
A-Rum Yoon, Pyung-Hwan Kim, Joo-Hyuk Sohn, Chae-Ok Yun, Joo-Hang Kim

**946**

*Combined E1B-Mutant Adenovirus and Radiotherapy Generates Synergistic Anti-Tumor Effects*  
Jaesung Kim, Jung-Hee Choi, Jing-Hua Huang, Pyung Hwan Kim, Beom-Seok Suh, Jin-Sil Seong, Chae-Ok Yun

**947**

*RNAi-Mediated Bcl-XL Depletion Sensitizes Pancreatic Cancer Cells to TNF $\alpha$  Induced Apoptosis*  
Jirong Bai, Mark P. Callery

**948**

*Regression of Established Melanoma Tumors through Intratumoral Delivery of HIV-1 Vpr Using In Vivo Electroporation*  
Andrea N. McCray, Chuanhai Cao, Karuppiah Muthumani, David B. Weiner, Kenneth Ugen, Richard Heller

**949**

*Intratumoral Adenovirus-Mediated Interferon Alpha Gene Transfer Induces Regional Direct Cytotoxicity and Possible Systemic Immunity Against Pancreatic Cancer*  
Kazunori Aoki, Masaki Ohashi, Yoshiaki Miura, Kimiko Yoshida, Shumpei Ohnami, Yukio Kitade, Teruhiko Yoshida

**950**

*FasL- Over-Expressing T Lymphocytes as Effector Cells in Anti-Prostate Cancer Gene Therapy*  
Julie C. Bielawski, Daniel H. Fowler, Jeffrey A. Medin

**951**

*Antitumor Activity of AAV-2 Vector Encoding Modified TRAIL Gene*  
Jinsang Yoo, Seeyoung Choi, Kyung-Sun Hwang, Won-Kyung Cho, Cho-Rok Jung, Dong-Soo Im

**952**

*Delivery of Tissue Inhibitor of Metalloproteinase 3 Inhibits Growth of Lung Cancer Tumours In Vivo*  
Katherine M. Finan, Greg Hodge, Ann M. Reynolds, Sandra J. Hodge, Mark D. Holmes, Andrew H. Baker, Paul N. Reynolds

**953**

*STAT5 Is Phosphorylated in Malignant Melanoma Leading to Proliferation Via Activation of ERK: Potential for Anti-Tumor Therapy*  
Alireza Mirmohammadsadegh, Walter Bardenheuer, Annett Baer, Sandeep Nambiar, Ulrich R. Hengge

**954**

*Ectopic Production of MDA-7/IL-24 Inhibits Invasion and Migration of Human Lung Cancer Cells*  
Isao Ito, Began Gopalan, Yuji Saito, Abner M. Mhashilkar, Sunil Chada, Rajagopal Ramesh

**955**

*Bystander Activity of Ad-md<sub>7</sub>: MDA-7 Protein Kills Melanoma Cells Via a Receptor-Dependent but STAT3-Independent Mechanism*  
Sunil Chada, Abner Mhashilkar, Rajagopal Ramesh, John B. Mumm, Dora Bocangel, Elizabeth A. Grimm, Suhendan Ekmekcioglu

**956**

*Suppression of Tumor Metastasis by Adenoviral-Mediated MRP-1/CD9 Expression*  
Yi Lu, Jun Zhang, Bogdan Ceacareanu, Aviv Hassid, Lisa K. Jennings

**957**

*bcl-xL Antisense Oligonucleotides as an Enhancer of the Cytotoxic Effects of Cisplatin, Mitomycin C and Paclitaxel in the Treatment of Bladder Cancer*  
Axel Schaaf, Sreedhar Sagi, Sigrun Langbein, Lutz Trojan, Peter Alken, Maurice S. Michel

**958**

*Rapid Blockade of Telomerase Activity and Tumor Cell Growth by Ribbon Antisense Oligodeoxynucleotides to hTR*  
Jong-Gu Park, Arun Bajpai

**959**

*bcl-2 Antisense Oligonucleotides Enhance the Cytotoxic Effects of Mitomycin C in the Treatment of Bladder Cancer*  
Axel Schaaf, Sreedhar Sagi, Sigrun Langbein, Lutz Trojan, Peter Alken, Maurice S. Michel

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

#### Targeted Cancer Therapies III

**960**

*Regulation of Cyclooxygenase-2 and Vascular Endothelial Growth Factor Promoter Based Conditionally Replicating Adenoviruses for Treatment of Cervical Cancer*

Anna Kanerva, Mari Raki, Gerd J. Bauerschmitz, Koichi Takayama, Masato Yamamoto, Renee A. Desmond, David T. Curiel, Akseli Hemminki

**961**

*E1B-55 kD-Deleted Adenovirus Driven by Murine Oct-3/4 Promoter for Bladder Cancer Therapy*

Chun-Fu Chang, Ai-Li Shiau, Gia-Shing Shieh, Chao-Liang Wu

**962**

*Hypoxia- and Radiation-Activated Cre/loxP Molecular Switch Vectors for Gene Therapy of Solid Tumors*

Olga Greco, Brian Marples, Michael C. Joiner, George D. Wilson, Simon D. Scott

**963**

*Development and Testing of Anti-Cancer Sleeping Beauty Transposons for Localized Tumors and Use in Metastasis Suppression*

John R. Ohlfest, Scott Perkinson, Paul Lobitz, Diane Hasz, Joel Frandsen, R. Scott McIvor, David A. Largaespada

**964**

*Infectivity Enhanced, hTERT Promoter-Based Conditionally Replicative Adenoviruses Are Useful for the Treatment of Small Cell Lung Cancer*

Koichi Takayama, Junji Uchino, Akiko Harada, Yosuke Kawakami, David T. Curiel, Yoichi Nakanishi

**965**

*A Novel Approach for Gene Transduction with Adenovirus/Fibrin Glue System*

Minori Sakurai, Tkeyuki Misawa, Hiroaki Shiba, Yohji Yamazaki, Katuhiko Yanaga

**966**

*Synergy between Angiostatin-Endostatin and Tie-2: A Novel Anti-Angiogenic Gene Therapy for Prostate Cancer*

Sudhanshu P. Raikwar, Chinghai Kao, Thomas A. Gardner

**Saturday, June 5**

**967**

*Angiotensinogen Inhibits Growth and Angiogenesis of Human Mammary Carcinoma and Prevents Pulmonary Metastasis of Murine Melanoma Cancer*

Céline Bouquet, Noel Lamande, Jean-Marie Gasc, Franck Griselli, Paule Opolon, Elisabeth Connault, Michel Perricaudet, Pierre Corvol

**968**

*A Dual-Action Armed Replicating Adenovirus for the Treatment of Bone Metastases of Breast Cancer*

James J. Cody, Gray R. Lyons, Joanne T. Douglas

**969**

*Transcription Controlled Gene Therapy Against Tumor Angiogenesis*

Shoshana Greenberger, Aviv Shaish, Eyal Breitbart, Israel Hodish, Livnat Bangio, Niva Yaakov, Iris Goldberg, Iris Barshack, David Wallach, Tanya Goncharov, Dror Harats

**970**

*A Fiber Modified SLPI Promoter Based Conditionally Replicating Adenovirus for Treatment of Ovarian Cancer*

Daniel T. Rein, Martina Breidenbach, Minghui Wang, Tyler O. Kirby, Gene P. Siegal, Gerd J. Bauerschmitz, Peter Dall, Dirk M. Nettelbeck, Akseli Hemminki, Ronald D. Alvarez, David T. Curiel

**971**

*New Generation Conditionally Replicative Adenovirus for Hormone-Refractory Prostate Cancer*

Tatyana Gavrikova, Julia Davydova, Long P. Le, Hidetaka A. Ono, Pedro J. Ramirez, Victor Krasnykh, David T. Curiel, Masato Yamamoto

**972**

*Gene-Modified Human Glioma Cells Producing Soluble Human Plasminogen Kringle 5 Peptide Suppress Brain Tumor Progression*

Sabrina R. Perri, Josephine Nalbantoglu, Borhane Annabi, Zafiro Koty, Richard Béliveau, Jacques Galipeau

**973**

*Efficient Transduction of Bone Marrow-Derived Mesenchymal Stem Cells for Cancer Gene Therapy Using a Tropism-Modified Adeno-Associated Virus (AAV) Vector*

Brett M. Hall, Amy E. Henning, Jeffrey S. Bartlett

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Saturday, June 5

**974**

*Adeno-Associated Viral (AAV)-Mediated Gene Transfer of Soluble Vascular Endothelial Growth Factor Receptor 3 (sVEGFR3) Inhibits Development of Lymph Node Metastases*

Jian-Min Lin, Alshad S. Lalani, Thomas Harding, Weiwei Wu, Bo Luan, Satya Yendluri, Melinda J. VanRoey, Yulong He, Kari Alitalo, Karin Jooss

**975**

*TSTA Mediated Multi-Modality Gene-Expression Imaging in Hormone Refractory Prostate Cancer*  
Makoto Sato, Mai Johnson, Liqun Zhang, Sanjiv S. Gambhir, Michael Carey, Lily Wu

**976**

*Decreased Tumorigenic Potential of EphA2-Overexpressing Breast Cancer Cells Following Treatment with Adenoviral Vectors That Express EphrinA1*

Loren W. Noblitt, Dinesh S. Bangari, Shruti Shukla, Deborah W. Knapp, Sulma Mohammed, Michael S. Kinch, Suresh K. Mittal

**977**

*Selective Transduction of Malignant Glioma by Lentiviral Vectors Pseudotyped with LCMV Glycoproteins*

Hrvoje Miletic, Yvonne Fischer, Harald Neumann, Volkmar Hans, Tsanan Gioglou, Martina Deckert, Dorothee von Laer

**978**

*Optimization of the Tet-on System To Regulate Interleukin 12 Expression in the Liver for the Treatment of Hepatic Tumors*

Maider Zabala, Lin Wang, Ruben Hernandez-Alcoceba, Wolfgang Hillen, Cheng Qian, Jesus Prieto, Maria-Gabriela Kramer

**979**

*The Gene Expression and Function of Adenoviral E3-Genes in Murine Tumour Cell Lines*

Richard J. Hill, Gunnell K. Hallden, Yaohe Wang, Jennelle Francis, Catherine Simpson, David H. Kirn, Nicholas R. Lemoine

**980**

*Evaluation of the Tumor Specificity of a Conditionally Replicative Adenovirus Controlled by a Modified Human Core Telomerase Promoter*

Sergia Bortolanza, Ruben Hernandez-Alcoceba, Gabriela Kramer, Jesus Prieto, Cheng Qian

**981**

*Gene Therapy Clinical Trials Worldwide 1989-2003: An Overview*

Michael L. Edelstein, Mohammad R. Abedi, Jo Wixon, Richard M. Edelstein

### Gene Regulation: Vectors

**982**

*Improved Transduction of Primary Murine Hepatocytes by Recombinant Adeno-Associated Virus 2 Vectors In Vivo*

Li Zhong, Weiming Li, Zuocheng Yang, Linyuan Chen, Yanjun Li, Keyun Qing, Kirsten A. Weigel-Kelley, Mervin C. Yoder, Weinian Shou, Arun Srivastava

**983**

*Human Derived Dual Reporter/Suicide Gene System for Simultaneous Pet Imaging of Independent Molecular-Biological Processes In Vivo*

Vilia Tourkova, Małgorzata Dabrowska, Michael Doubrovin, Inna Sereanova, Jelena Vider, Tatiana Beresten, Shangive Cai, Ronald Finn, Juri Gelovani, Tjuvajev, Vladimir Ponomarev

**984**

*Fetal Administration of Lentiviral Vectors and the Potential for Germ Cell Transduction in Rhesus Monkeys (*Macaca mulatta*)*

Chang I. Lee, Daniel F. Jimenez, Donald B. Kohn, Alice F. Tarantal

**985**

*Transposition of the Agouti Transgene by Sleeping Beauty Transposon Is Not Associated with Significant Changes in DNA Methylation in the Mouse Genome*  
C.-W. Park, D. Largaspada, B. T. Kren, C. J. Steer

**986**

*Efficient Gene Delivery to Human and Rodent Islets with Double Stranded (ds)-AAV2-Based Vectors*

Khaja K. Rehman, Zhong Wang, Rita Bottino, A. N. Balamurugan, Juan Li, Xiao Xiao, Massimo Trucco, Paul D. Robbins

**987**

*Highly Efficient Regulation of Gene Expression by Tetracycline in a Novel Replication-Defective Herpes Simplex Viral Vector*

Feng Yao, Chris Theopold, Daniela Hoeller

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Saturday, June 5

**988**

*Effect of Lentiviral Promoter and Fetal Route of Administration on Transgene Biodistribution and Expression in Rhesus Monkeys (*Macaca mulatta*)*  
Daniel F. Jimenez, Chang I. Lee, Cristin E. O'Shea, Donald B. Kohn, Alice F. Tarantal

**989**

*IAI.3B Promoter Is Ovarian Cancer Specific*  
Katsuyuki Hamada

**990**

*Transduction of Pancreatic Islets with Pseudotyped Adeno-Associated Virus: Effect of Viral Capsid*  
Nan Zhang, Nathalie Clement, Haojiang Zhang, Dongmei Chen, Shuang Fu, Michael Linden, Jonathan S. Bromberg

**991**

*Construction of a Novel Human Artificial Chromosome and Its Use for Gene Delivery*  
Motonobu Katoh, Fumiaki Ayabe, Satoko Norikane, Hidetoshi Hoshiya, Xianying Ren, Tetsuji Suda, Chiga Okita, Mitsuo Oshimura

**992**

*Lentiviral and Retroviral Vectors for Transcriptionally Targeted Expression of Human Beta-Globin*  
Annarita Miccio, Giulia Facchini, Francesco Lotti, Claudia Rossi, Giuliana Ferrari

**993**

*Activation of Gene Expression from Silenced Adenovector Genomes*  
Duncan McVey, Chi Hsu, Lisa Wei, C. Richter King, Douglas E. Brough

**994**

*Gene Knockdown in Mice by Using Lentiviral Vectors Expressing Small Interfering RNA*  
Oded Singer, Masahito Ikawa, Nien Hoong, Greg Campbell, Inder M. Verma

**995**

*Inducible Gene Expression by Use of an HET-Amplicon Vector*  
Alexandra Winkeler, Miguel Sena-Esteves, Norbert Galldiks, Lisa Li, Markus Klein, Parisa Monfared, Maria A. Rueger, Christof Knoess, Klaus Wienhard, Benedikt Rueckriem, Wolf D. Heiss, Andreas H. Jacobs

**996**

*Application of a Novel Human Artificial Chromosome (HAC) Vector to Gene Therapy Aimed at Erythropoietin (EPO) Replacement*  
Minoru Kakeda, Masaharu Hiratsuka, Keiko Nagata, Masayo Takahashi, Fumiaki Ayabe, Motonobu Katoh, Mitsuo Oshimura, Kazuma Tomizuka

**997**

*Specific Expression of Transgenes in Insulin-Secreting Islet Cells by Lentiviral Vectors*  
Andreas Hofmann, Dagmar Kress, Barbara Kessler, Eckhard Wolf, Alexander Pfeifer

**998**

*Evaluation of Promoter Elements for Specific Expression in Human Airway Epithelia Using a Self-Inactivating (SIN) Equine Lentivector*  
Angela M. Giddings, Lawrence Ostrowski, John C. Olsen

**999**

*Development of Orthogonal Multiplex Gene-Switch Systems Based on Engineered Ecdysone Receptors and Inducing Ligands Selected from a Single Chemotype*

Jennifer L. Friz, Beth Vertin, Christine S. Thompson, Subba R. Palli, Mohan Kuman, Orestes Chortyk, Andy Meyer, Tom Meteyer, Howard Smith, Robert E. Hormann, Dean E. Cress

**1000**

*Introns of the Angiotensin II Type 2 Receptor (AT2) Enhance Its Adenoviral-Mediated Over-Expression In Vitro*  
Hongwei Li, Yongxin Gao, Mohan K. Raizada, Colin Sumners

**1001**

*Use of cHS4 Chromatin Insulator To Improve Efficacy and Safety of Retrovirus Vectors*  
David W. Emery, Mari Aker, Tamon Nishino, Julie Tubb, George Stamatoyannopoulos

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

#### Clinical Gene Therapy

Room 200 A-C

**1002 9:00 AM**

*Immune Responses to AAV and to Factor IX in a Phase I Study of AAV-Mediated, Liver-Directed Gene Transfer for Hemophilia B*

Katherine High, Catherine Manno, Denise Sabatino, Sylvia Hutchison, Michael Dake, Mahmood Razavi, Robin Kaye, Valder Aruda, Roland Herzog, Pradip Rustagi, John Rasko, Keith Hoots, Phillip Blatt, Jurg Sommer, Margaret Ragni, Margareth Ozelo, Barbara Konkle, Ruth Lessard, Alvin Luk, Bertil Glader, Glenn Pierce, Linda Couto, Mark Kay

**1003 9:20 AM**

*HIV-1-Derived Lentivirus Vector-Based Antisense Gene Therapy: Towards an Alternative Treatment for HIV/AIDS*

Boro Dropulic, Laurent Humeau, Gwendolyn K. Binder, Xiaobin Lu, Vladimir Slepushkin, Randall Merling, Patricia Echeagaray, Mario Pereira, Tatiana Slepushkina, Scott Barnett, Lesia K. Dropulic, Richard Carroll, Bruce Levine, Rob Roy MacGregor, Carl H. June

**1004 9:40 AM**

*Integration Analyses in ADA-Deficient SCID Patients Successfully Treated with Stem Cell Gene Therapy*  
A. Aiuti, B. Cassani, F. Cattaneo, G. Andolfi, M. Mirolo, A. Recchia, L. Callegaro, A. Tabucchi, F. Carlucci, S. Slavin, R. Miniero, F. Mavilio, M. G. Roncarolo, C. Bordignon

**1005 10:00 AM**

*Expression of Normal Dystrophin Following Myoblast Transplantation to Duchenne Muscular Dystrophy Patients*

Jacques P Tremblay, Daniel Skuk, Bouchard Jean-Pierre, Michel Sylvain, Roy Raynald, Goulet Marilyne, Roy Brigitte, Pierre Chapdelaine, Dugré Francine, Jean-Guy Lachance, Louise Deschênes, Hélène Senay

**1006 10:20 AM**

*Preliminary Results of a Phase I Trial Using Retroviral Gene Transfer of G156A MGMT To Protect Hematopoiesis during BG and BCNU Therapy of Advanced Malignancies*

Jane Reese, Karen Lingas, Pamela Ksenich, Colin Sweeney, Omer Koc, Stanton Gerson

**Sunday, June 6**

**1007 10:40 AM**

*Safety of Adenoviral Vectors: Results of Clinical Investigations in 445 Cancer Patients Treated with Advexin® (Adenoviral p53) Gene Therapy*

Louis A. Zumstein, Donna Call, James Merritt, Robert E. Sobol, Kerstin Menander

**1008 11:00 AM**

*Intratumoral Injection of INGN 241, a Non-Replicating Adenovector Expressing the Melanoma-Differentiation Associated Gene-7 (MDA-7/IL-24): Biologic Outcome in Advanced Cancer Patients*

Alex W. Tong, John Nemunaitis, Dan Su, Yuan Zhang, Casey Cunningham, Neil Senzer, George Netto, Dawn Rich, Abner Mhashilkar, Karen Parker, Keith Coffee, Rajagopal Ramesh, Suhendan Ekmekcioglu, Elizabeth A. Grimm, Jill van Wart Hood, James Merritt, Sunil Chada

**1009 11:20 AM**

*A Phase I Study of the Clinical and Local Biological Effects of Intratumoral Injection of mda-7 (IGN 241) in Patients with Advanced Carcinoma*

C. Casey Cunningham, Sunil Chada, James Merritt, Alex Tong, Neil Senzer, Yuan Zhang, Abner Mhashilkar, Karen Parker, Sasha Vukelja, Donald Richards, Jill Hood, Keith Coffee, John J. Nemunaitis

#### Adenovirus: Toxicity and the Immune Response

Room 208 A-D

**1010 9:00 AM**

*Interference with the IL-1 Signaling Pathway Significantly Improves the Toxicity Profile of Systemically Applied Adenovirus Vectors*

Dmitry M. Shayakhmetov, Zong-Yi Li, Shaoheng Ni, Andre Lieber

**1011 9:15 AM**

*Tumor Specific Oncolytic Adenovirus Vector VRX-011 Which Overproduces ADP and Has the E4 Promoter Replaced by the hTERT Promoter*

Mohan Kuppuswamy, Ann E. Tollefson, Karoly Toth, Jacqueline F. Spencer, Konstantin Doronin, Drew L. Lichtenstein, Lou Zumstein, William S. M. Wold

**1012 9:30 AM**

*Ad Interactions with the Complement Systems of Humans and Mice*

Haixiang Jiang, Ruth Everett, Anne Kiang, Zhaoxia Wang, Huame Zhang, Delila Serra, Michael M. Frank, Andrea Amalfitano

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

**1013 9:45 AM**

*Complement Component 3 Facilitates Ad5-Mediated Liver Transfection*

Kurt R. Zinn, Tandra R. Chaudhuri, Amanda Stargel, Victor Krasnykh, Alex J. Szalai

**1014 10:00 AM**

*Helper-Dependent Adenovirus Vectors Elicit Intact Innate but Attenuated Adaptive Host Immune Responses In Vivo*

Daniel A. Muruve, Matthew J. Cotter, Anne K. Zaiss, Lindsay R. White, Trevor Chan, Sharon A. Clark, Joel Ross, Robert A. Meulenbroek, Gunhild M. Maelandsmo, Robin J. Parks

**1015 10:15 AM**

*Induction of Protective Immunity to Anthrax Lethal Toxin with a Chimpanzee Adenovirus-Based Vaccine Carrier in the Presence of Pre-Existing Anti-Human Adenovirus Immunity*

Masahiko Hashimoto, Julie L. Boyer, Neil R. Hackett, James M. Wilson, Ronald G. Crystal

**1016 10:30 AM**

*Novel Chimeric Adenovirus Vaccine Vectors*

Soumitra Roy, Yan Zhi, David Clawson, Guangping Gao, Gary P. Kobinger, James M. Wilson

**1017 10:45 AM**

*Metabolically Biotinylated Viruses for Vector Targeting, Virus Purification, and Capsid Imaging*

Samuel K. Campos, M. Brandon Parrott, Michael March, Wah Chiu, Michael A. Barry

**1018 11:00 AM**

*Immune-Competent Cotton Rat Animal Model for Evaluation of Oncolytic Adenoviruses*

Karoly Toth, Jacqueline F. Spencer, Ann E. Tollefson, Mohan Kuppuswamy, Konstantin Doronin, Drew L. Lichtenstein, William S. M. Wold

**1019 11:15 AM**

*Immune System Regulation of Transgene Expression in the Brain 2: Differential Increase of mRNAs Encoding for Interferon-Regulated, Chemokine, and T-Cell Genes during Either Innate Acute or Chronic Systemic Adaptive Immune Responses to First Generation Adenoviral Vectors*

Jeffrey M. Zirger, Carlos Barcia, Chunyan Liu, Mariana Puntel, Giovanni Siti, Luca Guidotti, Ngan Mitchell, Iain Campbell, Maria G. Castro, Pedro R. Lowenstein

**Sunday, June 6**

### Targeting Cancer Therapies

Auditorium Room 1

**1020 9:00 AM**

*Development and Evaluation of a Genetically-Modified Adenovirus Vector That Specifically Targets Tumors Via the Bloodstream Following Systemic Administration*

Masaki Akiyama, Peter W. Roelvink, David A. Einfeld, Selva Murugesan, C. Richter King, Thomas J. Wickham

**1021 9:15 AM**

*VP22 Mediates Tumor Vasculature Specific Targeting*

Sudhanshu P. Raikwar, Thomas A. Gardner, Chinghai Kao

**1022 9:30 AM**

*Ionizing Radiation-Induced Adenovirus Infection Is Mediated by Dynamin 2*

Jiong Yang, Eyad Abu-Isa, Theodore S. Lawrence, Ming Zhang

**1023 9:45 AM**

*Adenoviruses Transport across a Polarized Monolayer of Caco-2 Cells by Transferrin Receptor-Mediated Transcytosis*

Zeng B. Zhu, Sharmila K. Makhija, Baogen Lu, Minghui Wang, Meredith A. Preuss, Angel A. Rivera, Fen Zhou, Gene P. Siegal, Ronald D. Alvarez, David T. Curiel

**1024 10:00 AM**

*Dual Targeted Vector for Multiple Myeloma Tumors and its Associated Angiogenesis*

Louay K. Hallak, Jaime R. Merchan, Suzanne M. Greiner, Chris M. Storgard, Stephen J. Russell

**1025 10:15 AM**

*Efficiency, Specificity and Efficacy of Genetically-Modified Adenovirus Vector on Peritoneal Cancers*

Selva R. Murugesan, Masaki Akiyama, Peter W. Roelvink, David A. Einfeld, C. Richter King, Thomas J. Wickham

**1026 10:30 AM**

*Targeted Measles Virotherapy for Pancreatic Cancer*

Elizabeth M. Hadac, Kah-Whye Peng, Takafumi Nakamura, Laurence J. Miller, Stephen J. Russell

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Directory

Sunday, June 6

**1027 10:45 AM**

*A 4-Week Safety Study of Reolysin® Administered by Daily Intravenous Infusion to Cynomolgus Monkeys, with a 4-Week Recovery Period*

Ken Draper, Lydia Andrews-Jones, Jeremy Evans, Matthew C. Coffey, Brad Thompson

**1028 11:00 AM**

*Virus-Mediated RNAi for Skp-2 in Human Small Cell Lung Carcinoma Cells Resulted in the Inhibition of Tumor Cell Growth through the Increase of p27kip1 and p21*

Hidetoshi Sumimoto, Shizuko Yamagata, Ayako Shimizu, Makoto Miyagishi, Hiroyuki Miyoshi, Kazunari Taira, Yutaka Kawakami

**1029 11:15 AM**

*Increased Fluid Secretion after Adenoviral-Mediated Transfer of the Human Aquaporin-1 cDNA to Irradiated Minipig Parotid Glands*

Z. Shan, J. Li, C. Zheng, X. Liu, C. Zhang, C. M. Goldsmith, R. B. Wellner, B. J. Baum, S. Wang

### Optimization and In Vivo Monitoring of Cancer Suicide Gene Therapy

Auditorium Room 1

**1030 9:00 AM**

*Engineering Cytosine Deaminase for Suicide Gene Therapy*

Margaret E. Black, Sheri D. Mahan, Greg C. Ireton, Catherine Knoeber, Barry L. Stoddard

**1031 9:15 AM**

*Improved Anti-Brain Tumor Efficacy of  $\Delta\gamma134.5$  HSV Expressing Cytosine Deaminase*

James M. Markert, M. Brad Guffey, Debbie Della Manna, Donald J. Buchsbaum, Kathleen H. Price, Catherine P. Langford, G. Yancey Gillespie

**1032 9:30 AM**

*Massive Expression of a Suicide Gene Using a Replicative but Non-Disseminative Adenovirus for Treatment of Solid Tumors*

Denis Bourbeau, Cara Lau, Genevieve Lavoie, Anne-Marie Mes-Masson, Josephine Nalbantoglu, Bernard Massie

**1033 9:45 AM**

*A Novel Mechanism of Synergistic Cytotoxicity between Prodrugs, 5-FC and GCV in Double Suicide Gene Therapy*

Paul D. Boucher, Jessica O'Konek, Svend O. Freytag, Donna S. Shewach

**1034 10:00 AM**

*The Optimization of Cancer Gene Therapy Using the Measurement of Viral Gene Expression Magnitude and Distribution*

Kenneth N. Barton, Stephen L. Brown, Jae Ho Kim, Svend O. Freytag

**1035 10:15 AM**

*Syncytia Induction Enhances the Oncolytic Potential of Vesicular Stomatitis Virus in Virotherapy for Cancer*

Oliver Ebert, Katsunori Shinozaki, Chryssanthi Kournioti, Man-Seong Park, Adolfo Garcia-Sastre, Savio L. C. Woo

### Naked DNA-Based Gene Transfer

Room 205 A-D

**1036 9:00 AM**

*Effective Acceleration of Wound Healing by Simultaneous Transfection of Hepatocyte Growth Factor Gene and Prostacyclin Synthase Gene Using Shima Jet Yasuo Kunugiza, Naruya Tomita, Tetsuya Tomita, Hideo Hashimoto, Keita Yamasaki, Hiromi Koike, Hideki Yoshikawa, Ryuichi Morishita*

**1037 9:15 AM**

*Site-Specific Genomic Integration in Hematopoietic Cells*

Roger P. Hollis, Sarah Nightingale, Michele P. Calos, Gay M. Crooks, Donald B. Kohn

**1038 9:30 AM**

*Intramuscular Plasmid DNA Electrotransfer. Biodistribution, Degradation and Stability of the Transfectable Pool of Plasmid DNA*

Michel-Francis Bureau, Naimi Sadoui, Johanne Seguin, Daniel Scherman

**1039 9:45 AM**

*Mechanistic Insights into the Persistence of Non-Viral Mediated Gene Transfer In Vivo*

Efren Riu, Zan Huang, Mark A. Kay

**1040 10:00 AM**

*A Direct Comparison of Two Non-Viral Gene Therapy Vectors for Somatic Integration: In Vivo Evaluation of the Bacteriophage Integrase PhiC31 and the Sleeping Beauty Transposase*

Anja Ehrhardt, Hui Xu, Zan Huang, Mark A. Kay

**1041 10:15 AM**

*Treatment of Hyperbilirubinemia in the Gunn Rat by Intravascular Gene Transfer into Muscle with Naked Plasmid DNA (pDNA)*

Istvan Danko, Zhen Jia

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

Sunday, June 6

**1042 10:30 AM**

*RNA as a Source of Transposase for Sleeping Beauty - Mediated Transposition and Long-Term Expression in Somatic Cells and Tissues*

Andrew C. Wilber, Joel L. Frandsen, Lalitha R. Belur, Paul R. Score, Jennifer L. Geurts, David A. Largaespada, Perry B. Hackett, R. Scott McIvor

**1043 10:45 AM**

*Spontaneous Transfection of B Lymphocytes*

Mara Gerloni, Marta Rizzi, Paola Castiglioni, Matthew Wheeler, Maurizio Zanetti

**1044 11:00 AM**

*Growth Hormone Releasing Hormone Plasmid*

*Administration Decreases Tumor Growth and Enhances Immune Function in Nude Mice*

Amir S. Khan, Ingrid W. Anscombe, Kathleen K. Cummings, Melissa A. Pope, Louis C. Smith, Ruxandra Draghia-Akli

**1045 11:15 AM**

*Neonatal Tolerance Induction by Repetitive DNA Vaccination*

An-Bang Liu, Kuo-Feng Tai, Shinn-Zong Lin

### Gene Regulation: Animal Studies

Room 103 A-C

**1046 9:00 AM**

*Insertional Leukemogenesis after Retroviral Gene Transfer into Murine Hematopoietic Stem Cells*

Martijn H. Brugman, Monique M. A. Verstegen, Trudi P. Visser, Erna Fränel-Luiten, Manfred Schmidt, Albertus W. Wognum, Christof von Kalle, Gerard Wagenaar

**1047 9:15 AM**

*BXD Recombinant Inbred Mice Represent a Novel Immune Response Model to AAV-F.IX*

Huang-Ge Zhang, Hui-Chen Hsu, PingAr Yang, Qi Wu, Shaohua Yu, Jianhua Wang, Alexander Schlachterman, Katherine A. High, John D. Mountz

**1048 9:30 AM**

*Long-Term Regulated Gene Expression in Non-Human Primates Using a Single Non-Recombinogenic AAV Vector*

Victor M. Rivera, Lori Berk, Scott Wardwell, Hao Tang, Guang-ping Gao, Rebecca L. Grant, James M. Wilson, Tim Clackson

**1049 9:45 AM**

*Engineered ZFPs Can Prevent Limb Loss in Mice*

Jun Yu, Reed Hickey, Yan Huang, Edward Rebar, William C. Sessa, Frank J. Giordano

**1050 10:00 AM**

*Autoimmune Anemia in Macaques Following Erythropoietin Gene Therapy*

Pierre Chenuaud, Thibaut Larcher, Joe Rabinowitz, Nathalie Provost, Yan Cherel, Nicole Casadevall, Jude Samulski, Philippe Moullier

**1051 10:15 AM**

*A Fail-Safe System for Gene Therapy of Parkinson's Disease: Application of Removable Expression Cassette To Prevent Overproduction of Dopamine in a Rat Model*

Shin-ichi Muramatsu, Xiaog-gang Li, Takashi Okada, Mika Kodera, Yuko Nara, Naomi Takino, Kunihiko Ikeguchi, Fumi Urano, Hiroshi Ichinose, Imaharu Nakano, Keiya Ozawa

**1052 10:30 AM**

*Male Germline Cells Appear To Be at Risk Following Direct Injection Gene Transfer In Utero*

Paul J. Park, Joe Tellez, Graça Almeida-Porada, Esmail D. Zanjani, Christopher D. Porada

**1053 10:45 AM**

*Pharmacologically Regulated Regeneration of Functional Human Pancreatic Islets*

Gary P. Kobinger, Shaoping Deng, Jean-Pierre Louboutin, Marko Vatamaniuk, Victor M. Rivera, Franz Matschinsky, James F. Markmann, Timothy P. Clackson, Steven E. Raper, James M. Wilson

**1054 11:00 AM**

*Development of Adeno-Associated Virus (AAV) Vectors for Gene Therapy in the Mouse Model for Glycogen Storage Disease Type Ia (GSD-Ia)*

Dwight D. Koeberl, Baodong Sun, Luis Franco, Andrew Bird, Eric Faulkner, Y.-T. Chen

**1055 11:15 AM**

*Anterograde Delivery of Gene Product by rAAV5 Vectors*

Kevin D. Foust, Ronald J. Mandel, Paul J. Reier, Terence R. Flotte

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Abstract Directory

#### Neuro Gene Therapy II: New Approaches for Dominant, Recessive, Degenerative and Autoimmune Diseases

Room 211 A-D

**1056 9:00 AM**

##### *PhiC31 Integrase for Long-Term Non-Viral Gene Therapy in Rat Retina*

Thomas W. Chalberg, Hilary L. Genise, Douglas Vollrath, Michele P. Calos

**1057 9:15 AM**

##### *Successful Gene Therapy for Retinal Degenerations by Lentiviral Gene Transfer*

Nobushige Tanaka, Stylianos Michalakis, Martin Biel, Franz Hofmann, Inder M. Verma

**1058 9:30 AM**

##### *Adeno-Associated Virus Serotype 6 (AAV-6) Vector Mediated Gene Transfer of Soluble VEGF Receptors for the Treatment of Glioblastoma Multiforme*

Thomas C. Harding, Alshad S. Lalani, Jian Min Lin, Satyasri Yendluri, Bo Luan, Kathryn E. Koprivnikar, Guang Tu, Melissa A. Gonzalez-Edick, Wei Wei Wu-Prior, Melinda VanRoey, Byron Roberts, Richard LeCouter, Pete Dickinson, Karin Jooss

**1059 9:45 AM**

##### *The Lymphocytic Choriomeningitis Virus Envelope Glycoprotein Targets Lentivirus to Neural Progenitors in the Murine Brain*

Colleen S. Stein, Ines Martins, Beverly L. Davidson

**1060 10:00 AM**

##### *Long Term Expression of TPP-I in Rat and Non-Human Primate Brain Following Administration of AAV2CUhCLN2, a Candidate Gene Therapy Treatment for Late Infantile Neuronal Ceroid Lipofuscinosis*

Dolan Sondhi, Daniel A. Peterson, E. Lela Giannaris, Elizabeth Vassallo, Neil R. Hackett, Christine T. Sanders, Kimberly B. Bjugstad, John R. R. Sladek, Jr, Ronald G. Crystal, Both authors contributed equally

**Sunday, June 6**

**1061 10:15 AM**

##### *Specific and Efficient Transduction of the Cochlear Inner Hair Cells with Recombinant Adeno-Associated Virus Type 3 Vector*

Yuhe Liu, Takashi Okada, Tatsuya Nomoto, Kuniko Shimazaki, Kianoush Sheykholeslami, Shin-Ichi Muramatsu, Rahim Ajalli, Koichi Takeuchi, Hiroaki Mizukami, Akihiro Kume, Shuifang Xiao, Keiichi Ichimura, Keiya Ozawa

**1062 10:30 AM**

##### *Functional Rescue of Parkinsonian Non-Human Primates by a Dopamine Producing Multicistronic Lentiviral Vector*

Bechir Jarraya, Mimoun Azzouz, James Miskin, Scott G. Ralph, Fraser Wilkes, Jonathan Rohll, Lucy E. Walmsley, Fiona Ellard, Susan M. Kingsman, Kyriacos A. Mitrophanous, Philippe Hantraye, Stephane Palphi, Nicholas D. Mazarakis

**1063 10:45 AM**

##### *In Utero $\beta$ -Glucuronidase Gene Transfer with AAV Vectors Results in Widespread Transduction, Enzyme Activity, and Disease Correction in the MPS VII Mouse Brain*

Brian A. Karolewski, John H. Wolfe

**1064 11:00 AM**

##### *AdPEDF Therapy for Subfoveal Choroidal Neovascularization (CNV): Preliminary Phase I Results*

Timothy Stout, Michael Klein, Eric Holz, Anurag Gupta, David Saperstein, Lisa L. Wei, Angelita Ray, Paul Kessler, Robert N. Frank, Peter A. Campochiaro

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Aartsma-Rus, Annemieke	638	Almeida-Porada, Graça	1052	Arnold, Gregory S.	92
Abbosh, Phillip H.	452	Al-Shaykh Youssef, Eyas	934	Aronovich, Elena L.	824, 871
Abdel Latif, Mahmud	932	Altmann, Danny	720	Arroyo, Trini	445
Abd-El-Latif, Mahmoud	795	Alton, Eric	473, 480, 495, 496, 507, 720, 721, 935	Arruda, Valder	32, 103, 750
Abe, Tatsuya	650	Alvarez, Lucia	249	Arsenault, Jody R.	738
Abe, Tomio	190, 933	Alvarez, Ronald D.	443, 620, 623, 970, 1023	Arsic, Nikola	197, 236
Abedat, Suzane	932	Alvarez, Xavier	340	Aruda, Valder	1002
Abedi, Mohammad R.	981	Alvarez-Vallina, Luis	740	Arya, Suresh K.	71
Abmayer, Simone	243	Alvira, Mauricio R.	88	Asada, Hidetsugu	262, 348, 584
Abordo-Adesida, Evelyn	661	Amalfitano, Andrea	339, 543, 699, 862, 869, 1012	Asano, Ryutaro	63
Abruzzese, Ronald V.	139	An, Dong Sung	887	Asano, Shigetaka	582, 672, 910
Abu-Isa, Eyad	1022	An, Haesook	407	Aseguinolaza, Gloria G.	270
Acres, Bruce	577	Anai, Satoshi	845	Aslan, Hadi	827
Adamopoulou, Eleni C.	562	Andaluz, Anna	173	Aslanidi, George	420
Adams, Lisa C.	294, 627	Andarini, Sita	117	Ataa, Mohammad M.	794
Adler, Rima	708	Anderson, Bambi D.	610	Atanaskova, Natasha	62
Afik, Daniel	921	Anderson, Joseph S.	410	Atkinson, Mark A.	169, 250, 429, 565
Agadjanian, Hasmik	844	Andersson, Annika	825	Attila, Kovacs	695
Agarwal, Anurag	513, 671	Andersson, Karin	663	Attra, Heather	820
Ageyama, Naohide	703	Ando, Dale G.	582	Aubourg, Patrick	225
Agostini, Silvia	365	Andolfi, G.	1004	Auclair, Christian	909
Agrawal, Nalini	800	Andrade-Gordon, Patricia	750	Auricchio, Alberto	218, 647
Agudo, Judith	173	Andrassy, Martin	468	Austin, Melissa	539
Aguila, Alian	618	Andre-Schmutz, Isabelle	706	Avegilla, Scott	196
Aguirre, Gustavo	855, 857	Andrews, Robert	301	Avina, Maria D.	635
Ahman, Frederick	577	Andrews-Jones, Lydia	1027	Ayabe, Fumiaki	991, 996
Ahmed, Atique	451	Andrianaivo, Fanjam	140	Ayesh, Basem	30, 280
Aigrain, Yves	504	Anegon, Ignacio	97, 329	Ayesh, Suhail	30, 280
Aissaoui, Abderrahim	504	Anguela, Xavier M.	155	Ayuso, Eduard	173
Aitken, Moira L.	508	Anisimova, Valentina A.	843	Azoulay, Tamar	827
Aiuti, A.	1004	Anklesaria, Pervin	489, 881	Azuma, Junya	191
Aiuti, Alessandro	906	Annabel, Fu	695	Azzouz, Mimoun	235, 528, 532, 735, 1062
Ajalli, Rahim	1061	Annabi, Borhane	972	Bach, Patricia	560
Aker, Mari	1001	Anne, Hennig	695	Backstrom, Kristin	697
Akiyama, Masaki	1020, 1025	Annick, Brandenburger	684	Badens, Catherine	805
Akkina, Ramesh	410, 731	Annoni, Andrea	631	Baer, Annett	953
Al-Allaf, Faisal	36	Ansari, Aslam	834	Bagdanova, Anna	925
Albelda, Steven M.	572	Anscombe, Ingrid W.	1044	Bagg, Adam	341
Al-Dhalimy, Muhsen	873	Anson, Donald S.	66	Bahler, Cindy K.	335
Aldrich, Wayne	124, 619, 664	Aoki, Kazunori	949	Bahner, Ingrid	416, 545
Aleman, Tomas S.	218	Aoki, Motokuni	191	Bai, C. X.	499
Alexander, Ian E.	405	Appleby, Clare E.	204	Bai, Jirong	947
Alexander, Richard	65	Arakawa-Hoyt, Janice	192	Bai, Lin	468
Alferiev, Ivan S.	125, 200, 668	Arbetman, Alejandra E.	189	Bai, Yuan-Son	910
Ali, Robin R.	36	Arbuthnot, Patrick	553	Bai, Yuansong	672
Ali, Robyn R.	166	Arcangeli, Caterina	365	Bailey, Kent R.	306
Alipui, D. Olivier	372	Archer, David	399, 912	Bajgelman, Marcio C.	85
Alison, Malcolm	166	Arfi, Audrey	870	Bajpai, Arun	958
Alitalo, Kari	389, 974	Ariga, Tadashi	175	Baker, Andrew H.	127, 391, 666, 952
Aljamali, Majed N.	32	Arii, Shigeki	406	Baker, Kate	340
Alken, Peter	184, 957, 959	Armeanu, Sorin	9, 590	Baker, Kim	516, 541
Allaf, Faisal A.	235	Armentano, Donna	854	Bakker, Andrew	40
Allen, Cornell	198, 583	Armour, Gary	474	Bakker, Conny	784, 872
Allen, James M.	243, 718			Balaban, Alexandru T.	477
Allen, Paul	380			Balamotis, Michael A.	112, 300
Allen, Tara L.	881			Balamurugan, A. N.	986
Alley, Michael C.	618			Balloul, Jean-Marc	252

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Baltunis, Jacqueline	941	Bellinger, Dwight	34	Birman, Tatiana	30, 280
Balyasnikova, Irina V.	664	Bellini, William J.	554	Birney, Yvonne	109
Ban, Hiroshi	721, 723	Bellone, Matteo	350	Birraux, Jacques	170
Banerjea, Akhil	410, 731	Bellu, Anna Rita	153	Bishop, Kathie	539
Banerjee, Papia T.	124, 267	Belousova, Natalya	122	Bishop, Kathie M.	527
Bangari, Dinesh S.	976	Beltram, Fabio	365	Bitzer, Michael	9, 590, 742
Bangio, Livnat	969	Belur, Lalitha R.	871, 1042	Bizouarne, Nadine	577
Barbon, C. M.	694, 853	Benhamida, Sonia	225	Bjugstad, Kimberly B.	1060
Barcena, Alicia	192	Bennett, Jean	218	Black, Margaret E.	1030
Barcia, Carlos	459, 661, 1019	Bennett, Michael J.	147	Blackwell, Jerry L.	620, 664
Barcova, Maria	68	Bennett, Ryan	826	Blankinship, Michael J.	433, 718
Bardenheuer, Walter	953	Ben-nun-Shaul, Orly	795	Blatt, Phillip	1002
Bardin, Florence	54	Benrezzak, Nasro	97	Blau, C. Anthony	53, 299, 301
Barker, Shannon	240, 460	Benvegnu, Thierry	481	Blechacz, Boris R. A.	684
Barnes, Mack N.	620	Bercury, S.	694	Blissard, Gary W.	688
Barnett, Scott	1003	Bercury, S. D.	853	Blits, Bas	518, 519
Barratt-Boyes, Simon	554	Beresten, Tatiana	983	Blomley, Martin J.	935
Barret, Robin	589	Berg, Kristian	669	Bloquel, Carole	883
Barret-Boyes, Simon	778	Bergami, Alessandra	46	Blum, David	641
Barroga, Charlene F.	397	Bergert, Elizabeth R.	29	Boachie-Adjei, Oheneba	888
Barry, Michael A.	111, 131, 454, 894, 1017	Berges, Bradford	217, 223	Bobik, Thomas A.	436
Barshack, Iris	969	Berk, Arnold J.	773	Bocangel, Dora	955
Bartlett, David L.	65	Berk, Lori	215, 1048	Böckmann, Miriam	622, 943
Bartlett, Jeffrey S.	92, 109, 745, 973	Berlinghoff, Simone	344	Boczkowski, David	588
Barton, Kenneth N.	1034	Bernal, Giovanna	232	Bodine, David M.	902
Bartsevich, Victor	51, 302	Bernard, Robert M.	145	Boeffard, Françoise	97
Bartus, Raymond	227, 527, 536, 539	Bernloehr, Christian	9	Boelhauve, Marc	660
Barzon, Luisa	597	Bernhausen, Alexandra	440, 612	Boissier, Christophe	883
Basso, Luisa	870	Berraondo, Pedro	319	Boivin, Gregory P.	457
Bates, Paul	732	Bertani, Ilaria	41	Boller, Klaus	560
Bates, Susan	618	Bertin, Terry K.	159	Bonadio, Jeffrey	180
Batra, Raj K.	686	Bertolotti, Roger	710	Bondanza, Attilio	350
Battaglia, Anna	208	Bertoni, Carmen	810	Bongorno, Jennifer A.	437
Battaglia, Manuela	631	Bertucci, Donna	514	Bonini, Chiara	350, 398
Battini, Lorenzo	733	Bessis, Natacha	883	Bonnet, Dominique	166
Battmer, Karin	633	Bessodes, Michel	475	Bonsted, Anette	669
Bauer, Gerhard	259, 411, 606	Betz, Oliver B.	891	Bonton, Erik	234
Bauerschmitz, Gerd J.	442, 960, 970	Betz, Volker M.	891	Bordignon, C.	1004
Baum, B. J.	1029	Beunkens, Caroline	577	Bordignon, Claudio	41, 906
Baum, Bruce J.	769	Bharadwaj, Rikki	402	Borellini, Flavia	897
Baus, James	470	Bhardwaj, Shalini	389	Bortolanza, Sergia	980
Bauzon, Maxine	238	Bianco, Richard W.	200	Bory, Jean-Pai	252
Beagles, Karen	412	Bible, E.	42	Bosch, Assumpcio	173
Beard, Brian C.	1, 913	Biel, Martin	1057	Bosch, Fatima	155, 173
Beard, Georgina L.	168	Bielawska, Alicja	676	Bosma, Peter J.	130, 784, 872
Beattie, Stuart G.	875	Bielawski, Julie C.	950	Bossow, Sascha	9
Beaudet, Arthur	159, 163, 174, 789	Bieler, Alexa	612	Bosticardo, Marita	907
Beckett, Travis	691	Bienemann, Alison	636	Bottino, Rita	986
Beerens, Antoine M. J.	716	Bierhaus, Angelika	468	Boucher, Helene	918
Beeri, Ronen	932	Biessen, Eric	120	Boucher, Paul D.	1033
Beetz, Maarten	40	Biffi, Alessandra	41	Boudreau, Ryan	207
Beguinot, Francesco	647	Bigey, Pascal	883	Bouganim, Nathaniel	349
Behr, Jean Paul	471	Bigger, Brian W.	36, 166, 235	Bouis, Diane	153
Belenchia, Gretchen	340	Bilbao, Roberto	382	Boulanger, Pierre	663
Béliveau, Richard	972	Binder, Gwendolyn K.	6, 1003	Boulis, Nicholas M.	45, 439, 532, 735
Bell, Jason B.	824, 871	Binkley, Erin	515	Bouquet, Céline	967
Bell, Peter	692	Bird, Andrew	339, 699, 867, 1054	Bourbeau, Denis	1032

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Bourget, Lucie	135	Brugman, Martijn H.	1046	Cannon, Paula	696
Bourgoin, Christophe	870	Brunetti, Nicola	462	Cannon, Paula M.	68
Bouthillier, Francois	774	Brunetti-Pierri, Nicola	163, 174, 789	Cao, Chuanhai	948
Bouxsein, Mary L.	891	Brunmark, Charlott	894	Cao, Ou	33, 185
Boven, Epie	288	Bu, Jie	210	Capacio, Vanessa L.	652
Bovolenta, Chiara	375	Buchholz, Christian J.	333, 560, 614, 740	Caperton, Caroline V.	518
Bowden, Ramsay	480	Buchsbaum, Donald J.	674, 1031	Caplice, Noel M.	199, 392
Bowers, William J.	43, 211, 226	Buckley, Suzanne M. K.	36, 235	Carbonaro, Denise	177, 696, 905
Bowles, Dawn E.	17, 19, 91	Buening, Hildegard	18, 95	Carbonell, D.	702
Bowman, Katherine	828	Bueren, Juan A.	916	Cardoza, Dawn M.	273
Boyd, A. Christopher	479, 480	Bukreyev, A.	687	Carette, Jan E.	59, 121, 594
Boyd, Chris	496	Bull, David A.	193	Carey, Dee	174
Boyer, Julie	372, 558, 567, 570, 1015	Bullinga, Linda	80	Carey, Michael	975
Boyer, Olivier	459	Bunge, Mary	518, 519	Carime, Rodney M.	104
Boysen, Megan M.	461	Buning, Hildegard	435	Cario-Andre, Muriel	701
Boyton, Rosemary	720	Bunnell, Bruce A.	340, 514, 540	Carlucci, F.	1004
Brachman, Erin	305	Bureau, Michel-Francis	506, 1038	Carmona, Sergio	553
Brady, James	67, 728	Burgess, Shawn	13, 817	Carnes, Aaron	818
Brady, Roscoe O.	863	Burghes, Arthur H.	528	Caron, Nicolas J.	152, 239
Brambilla, Elena	46	Burnight, Erin R.	688	Carpenter, Matthew	513
Brambilla, Riccardo	41	Burns, Barry J.	420	Carpenter, Robert H.	273
Branch, Cynthia D.	500	Burstein, Haim	99, 880	Carpentier, Stephane	868
Branden, Lars J.	150	Burton, Dennis R.	542	Carrabino, Salvatore	837
Brandon, Eugene P.	227	Bushell, Andrew	352	Carrier, Ewa	522
Bras, Johannes	289	Butler, E. Brian	291	Carrión, Miguel	57
Braun, S. E.	546	Butti, Erica	46	Carroll, Miles W.	736
Braun-Falco, Markus	435	Button, B.	687	Carroll, Pamela M.	309
Breakefield, Xandra	23, 64	Buursma, Anne Rixt	716	Carroll, Richard	1003
Breese, George R.	50	Byrne, Barry J.	250, 251, 377, 565, 761, 859	Carter, Barrie	489, 754, 760
Breidenbach, Martina	623, 970	Byrne, Patrick S.	605	Cartier, Nathalie	225
Breitbart, Eyal	969	Byrnes, Andrew P.	464	Casadevall, Nicole	1050
Brem, Gottfried	660	Byun, Jonghoe	903	Casales, Erkuden	576
Bremmer-Bout, Mattie	638	Cadet, Mamonyj	475	Casanova, Jean-Laurent	907
Brenner, Malcolm K.	356	Cahill, Paul	109	Casavant, Thomas L.	4
Brenner, Michael	286	Cai, Shangive	258, 983	Case, Casey	107, 302
Brenner, Sebastian	915	Cai, Shijie	206	Case, Casey C.	51, 325, 531, 628, 649, 659, 901, 931
Breul, Astrid	142	Caillaud, Catherine	870	Cashion, Linda	791
Brigitte, Roy	1005	Calcedo, Roberto	88, 90, 128, 341	Cashman, Siobhan	515
Brill, Thomas	573	Caldwell, David	282	Casnici, Claudia	149
Brill-Almon, Einat	921	Caldwell, Lanise	176	Cassani, B.	1004
Brittan, Mairi	36	Cali, Simona	149	Castagliuolo, Ignazio	597
Brittin, Robin	937	Call, Donna	1007	Castiglioni, Paola	564, 1043
Bromberg, Jonathan S.	990	Callegaro, L.	1004	Castro, Maria G.	459, 661, 790, 1019
Bronstein, Michal	795	Callery, Mark P.	947	Catana, Andrea	677
Brooks, Alan	238, 791	Calmels, Boris	707, 708	Cataniag, Floro	422, 424
Brooks, David C.	37	Calos, Michele P.	152, 809, 810, 811, 814, 1037, 1056	Catron, Daniel J.	611
Brooks, Gabriel	284	Camire, Rodney M.	32	Cattaneo, F.	1004
Brosnan, M. Julia	391	Campbell, Adam G.	376, 893	Cattaneo, Roberto	24, 328
Brotchi, Jacques	641	Campbell, Greg	994	Caussin, Annabelle	487
Brough, Douglas E.	57, 516, 541, 993	Campbell, Iain	1019	Cavanagh Kyros, Julie A.	854
Broussau, Sophie	70	Campbell, Jacquelyn A.	111	Cavazzana-Calvo, Marina	706
Brouwer, Eric	114	Campbell, Kevin	251, 491	Cazzaniga, Sabrina	398
Brown, Lamar	527	Campbell-Thompson, Martha	169, 429	Ceacareanu, Bogdan	956
Brown, Patricia A.	139	Campochiaro, Peter A.	57, 1064	Cela, Racel	462
Brown, Stephen L.	1034	Campos, Samuel K.	1017	Cela, Racel G.	643
Bruder, Joseph T.	57, 689	Candotti, Fabio	175, 904, 907	Cerullo, Vincenzo	386

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Cervelli, Tiziana	16	Chen, Shu	292, 690	Chrriorini, John A.	890
Cervenak, Judit	908	Chen, Xinhua	844	Chris, Evans H.	892
Cesana, Daniela	3	Chen, Y.-T.	339, 699, 1054	Christensen, Lane	193
Chabannon, Christian	54, 743, 805	Chen, Yen-Ju	147	Christian, Lindsey	44
Chacur, Maru	530	Chen, Yong	82, 357	Chtarto, Abdelwahed	641
Chada, Sunil	500, 675, 954, 955, 1008, 1009	Chen, Yong Hong	93	Chuah, Marinee	413
Chae, Soo Yeong	453	Chen, Zhi-Ying	141	Chuah, Teong L.	596
Chai, Ning	732	Chen, Ziping	6	Chuang, Yao-Chi	884
Chalberg, Thomas W.	814, 1056	Chen, Zongyu J.	154, 216	Chung, Chris	298
Chamberlain, Jeffrey	237, 240, 241, 242, 243, 379, 433, 460, 718	Cheng, Cheng	5	Chung, Leland W. K.	604
Chan, Kitty P. Y.	679	Cheng, Jiin-Tsuey	637	Ciccarelli, Michele	386
Chan, Lawrence	39	Cheng, LiTeng	730	Cichutek, Klaus	333, 560, 614, 740
Chan, On Ying	132	Cheng, S. H.	694, 853	Cideciyan, Artur V.	218
Chan, Rebecca J.	52, 303, 342	Cheng, Seng	473, 507	Cieciuch, Adam	488
Chan, Trevor	1014	Cheng, Seng H.	465, 494, 854	Cioffi, William	174
Chan, Wan-Ching	162	Cheng, Wing-Shing	626	Clackson, Timothy	215, 301, 1048, 1053
Chancellor, Michael B.	884	Chenuaud, Pierre	1050	Clark, Heather	873
Chandler, Simon	931	Cherel, Yan	1050	Clark, Jocelyn N.	937
Chang, Alex H.	72, 652	Cherry, M.	694, 853	Clark, K. Reed	346, 744
Chang, Benjamin	278	Chesbro, A. L.	546	Clark, Karl	165
Chang, Benny	39	Chiang, Po-Hui	884	Clark, Sharon A.	1014
Chang, Chien-Wen	193	Chien, Sylvia	301	Clarke, Christian	643
Chang, Chun-Fu	961	Chilian, William M.	738	Clawson, David	128, 1016
Chang, Heesoon	104, 821	Chillon, Miguel	173	Clemens, Paula R.	248, 382
Chang, Jin Woo	757	Chillon, Miquel	155	Clement, Nathalie	990
Chang, Soo-Ik	821	Chinnasamy, Dhanalakshmi	76	Clift, Shirley	582
Channon, Keith M.	206, 391	Chinnasamy, Nachimuthu	76	Cloutier, Denise	251
Chapdelaine, Pierre	152, 1005	Chiocca, E. A.	796, 802	Coates, Allan	685
Chappell, James D.	111	Chiocca, E. Antonio	624, 804	Coccoris, Miriam	269
Charron, Catherine E.	874, 877, 878, 879	Chiorini, John A.	229, 230	Cockrell, Adam S.	69
Chatterjee, Saswati	393	Chipchase, Daniel	83	Cody, James J.	968
Chattopadhyay, Munmun	219	Chiriva-Internati, Maurizio	257, 587	Coffee, Keith	1008, 1009
Chaudhuri, Arabinda	682	Chiu, Wah	1017	Coffey, Matthew C.	1027
Chaudhuri, Tandra R.	1013	Chiu, Ya-Hui	43	Coffin, Rob	265, 266, 274
Chavez, Raymond A.	233, 530	Chiuchiolo, Maria	570	Cohen, Justus B.	63
Chen, Baosheng	468	Chiyo, Masako	571	Cohen-Haguenauer, Odile Y.	909
Chen, Changpo	833	Chmielnicki, Eva	571	Colben, Gail	445
Chen, Chang-Po	841	Cho, Gerald	545	Cole, Caroline	23
Chen, Chien-lun	449	Cho, Jean	71	Coleman, Trey	856
Chen, Chun-Liang	346, 744	Cho, Kyung-Ju	581	Colin, McKerlie	685
Chen, Cindy	826	Cho, Sungae	581	Colla, Joseph	358
Chen, Dongmei	990	Cho, Sung-Rae	224	Collen, Désiré	120, 387, 413, 563
Chen, Eric Y.	354	Cho, Won-Kyung	951	Collie, David	144, 496
Chen, Haifeng	37, 418	Choi, Donghoon	821	Collins, Holly A.	210
Chen, Helen H. W.	595	Choi, Il-Kyu	193	Collins, Margaret H.	294
Chen, Irvin S. Y.	887	Choi, Jung-Hee	444	Collins, P. L.	687
Chen, J.	499	Choi, Myung Min	946	Colosi, Peter	37, 89, 418, 752
Chen, Jian	640, 644	Choi, Seeyoung	951	Combee, Lynn A.	101
Chen, Jiang	468	Choi, Uimook	176, 915	Compans, Richard W.	730
Chen, Judy	316	Choi, Vivian	11	Campbell-Yhompson, Martha	106
Chen, Kui	431	Choi, Young-Kook	429, 761	Conese, Massimo	837
Chen, Linyuan	342, 982	Chong, Heung	352	Conlon, Thomas	98, 377, 875
Chen, Linyuann	303	Chorny, Michael	125, 668	Conlon, Thomas James	436
Chen, Minghan	672	Chortyk, Orestes	999	Connault, Elisabeth	285, 967
Chen, Ping	57	Chou, Cheng-Yang	595	Connolly, Jeanne M.	200
Chen, Q.	521	Chowdhury, Wasim H.	137	Contag, Christopher H.	810
				Converse, Andrea D.	7

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Author Index

Cook, Terence H.	36	Currier, Mark A.	294, 627	Deasy, Bridget M.	245, 246
Cook, Terrence	235	Curtin, James	790	DeBenedetti, Arrico	621
Coombes, R.	274	Cuturi, Maria C.	329	Deckert, Martina	977
Cooney, Ronan	776	Czerniak, Abraham	280	Dehari, Hironari	189, 926, 933, 938
Cooper, Gregory M.	385	Dabrowska, Małgorzata	983	Del Carro, Ubaldo	41
Cooper, J.	42	Dabrowska, Małgorzata	258	Dela Figuera, Nathalia	475
Cooper, Mark J.	501, 505, 849	Dai, Hui	357	Delepine, Pascal	479, 481
Cordelier, Pierre	370	Daigle, Bernie	817	Deleuze, Virginie	506
Corrales, Gaelle	518	D'Aiuto, Leonardo	554	Delgado, Christina	585
Corsi, Karin	895	Dake, Michael	1002	Della Manna, Debbie	1031
Corvol, Pierre	967	Daley, George Q.	923	DeMarco, Richard	65
Cosset, Francois-Loic	398	Dall, Peter	442, 970	Dermeneix, Barbara A.	471
Cossette, Travis L.	98	Dam, Wendy	153	den Dunnen, Johan T.	638
Costanzi-Strauss, Eugenia	85, 417	Dan, Qinghong	635	Denby, Laura	127
Cote, Colette A.	712	Dang, Y.	687	Denes, Bela	807
Cotrim, Ana Paola	769	Daniels, Gregory	352	Deng, Shaoping	1053
Cotter, Matthew J.	455, 1014	Daniels, Jan	108	Denham, Jerrod	232
Cotugno, Gabriella	647	Danilov, Sergei M.	391, 664	Dent, Carolyn	324, 531, 649
Courtman, David W.	198	Danko, Istvan	1041	Dermody, Terence S.	111, 121
Coutelle, Charles	36, 166, 235, 487	Dapas, Marina	197	Deschênes, Louise	1005
Couto, Linda	37, 102, 103, 753, 1002	Date, Isao	47, 205	Desmond, Renee A.	960
Cradick, Thomas J.	309	Dauty, Emmanuel	678	Desnick, R. J.	853
Craft, Timothy P.	898	Davern, Timothy J.	748	Devaux, Patricia	328
Cramer, Steve J.	134	Daviau, Judy	917	DeVeau, Ian F.	897
Cranston, Aaron N.	943	Davidson, Beverly L.	4, 80, 93, 171, 207,	Di Nunzio, Francesca	398
Cresawn, Kerry O.	250, 859	209, 229, 230, 491, 717, 1059	209, 829, 832	Di Paolo, Nelson C.	260, 271
Crescenzo, Dreg D.	135	Davies, Lee A.	143	Diamond, Scott L.	498
Cress, Dean E.	813, 999	Davies, Lee L. A.	144	Diaz, Rosa M.	23, 352, 741
Crettaz, Julien Swen	319	Davis, Alan R.	785	Dickinson, Pete	1058
Cripe, Timothy P.	294, 627	Davis, Bryce H.	927	Didier, Pete	340
Cristiana, Richard J.	359	Davis, Clay	801	Dijkhuizen, Paul	40, 423
Critchfield, Jaime	717	Davis, Mark E.	363	Dijon, Marilyne	743
Cromblehome, Timothy M.	56	Davis, Michael	45	Ding, Wei	754, 756
Cronin, James	533	Davis, Pamela B.	358, 829, 832	Dinsart, Christiane	684
Crooks, Gay M.	1037	Davydova, Julia	782, 786, 788, 971	DiPaolo, Nelson C.	276
Cruz, Pedro	530	Day, Chi-Ping	295	DiPersio, John F.	259, 606
Crystal, Ronald G.	160, 196, 338, 372, 374, 432, 488, 543, 557, 558, 567, 570, 670, 689, 711, 755, 888, 1015, 1060	Dayao, Jonathan	82	DiPietro, Martha A.	313
Cui, Feng-De	584	d'AZzo, Alessandrada	234, 698	Dmitriev, Igor P.	782, 786
Cullen, Karyn	855, 857	D'Costa, Jenice	71	Dobrzynski, Eric	33, 185
Cullen, Kristen	855, 857	De, Bishnu P.	338	Dodge, James C.	210
Cullis, Pieter R.	314	De Claro, R. Angelo	301	Doe, Barbara	556
Cummings, Kathleen K.	139, 1044	De Geest, Bart	120, 387, 563	Doherty, Ann	479, 480
Cummins, James H.	930	de Groot, Nathan	30, 280	Dolgilevich, Svetlana M.	466
Cunningham, C. Casey	1009	De Lagausie, Pascal	504	Domansky, Karel	850
Cunningham, Casey	1008	de las Alas, Maida M.	145	Dominiczak, Anna F.	391
Cunningham, Janet	215	de Leeuw, Bertie H. C. G. M.	286	Donahee, Mary H.	100
Cunningham, John M.	179	De Luca, Michele	182	Donahue, Kevin	394
Cunningham, Justine J.	227	De Palma, Michele	2, 41	Donahue, Robert E.	705, 707
Cunningham, Sara M.	105	de Verneuil, Hubert	701	Dong, Jian-Yun	676
Cunningham, Scott E.	19, 91	de Vries, Erik F. J.	716	Donovan, Maureen D.	502
Curiel, David T.	21, 28, 115, 116, 122, 124, 126, 129, 267, 289, 293, 320, 442, 443, 620, 623, 629, 640, 644, 662, 664, 782, 786, 788, 960, 964, 970, 971, 1023	de Winter, Christa L.	638	Dorigo, Oliver	773
		de Witte, Moniek	269	Doronin, Konstantin	447, 1011, 1018
		de Zanger, Ronald	387	Doubrovin, Michael	258, 983
		Dean, David A.	203, 812	Douglas, Joanne T.	126, 968
		Dean, Phillip	935	Doyennette, Marie Agnes	924
		Dean, Phillip	473	Doyle, Timothy	810

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Dragana, Milosevic	26	Emery, David W.	1001	Fehling, Hans Joerg	922
Draghia-Akli, Ruxandra	139, 273, 1044	Engelhardt, John F.	12, 754, 756, 760	Fei, Du	607
Dragos, Rachel	160, 374	Engels, Barbara	545	Feldmann, Frederik	373
Draper, Ken	1027	Engesaeter, Birgit	669	Feldmann, Heinz	368, 373
Dreicer, Robert	577	Engler, Jeffrey A.	811	Fellenberg, Kurt	916
Dreja, Hanna S.	724	Epperly, Michael W.	513, 671, 673	Feng, Lan	215
Driessens, Anita	204	Epstein, Alberto	805	Ferec, Claude	316, 479, 481
Dritschilo, Anatoly	617	Erbacher, Patrick	471, 836	Ferguson, Cole	708
Dropulic, Boro	6, 1003	Erger, Kirsten Elizabeth	436	Fernandez, Javier	539
Dropulic, Lesia K.	1003	Erickson, David C. C.	871	Ferrara, Luciana	317
Drosten, Matthias	622, 943	Ertl, Hildegund C. J.	37, 719	Ferrari, Aldo	365
Drury, Miya	315	Escudero, Brian	686	Ferrari, Giuliana	182, 403, 914, 992
D'Souza, Gerard G. M.	683	Escudero Garcia, Sara	507, 720	Ferrari, Stefano	182, 473, 720
Du, Lily M.	919	Essand, Magnus	626	Ferry, Nicolas	329
Du, Xiaohan	542	Essner, Jeff	165	Fetterolf, Christine	214
Duan, Dongsheng	15, 247	Esuvaranathan, Kesavan	264	Fichou, Yann	316
Ducharme, Marie-Eve	239, 537	Etingov, Igor	801	Fields, Carl R.	878
Duffey, Dianne C.	466	Eto, Yoshikatsu	858	Fieschi, Claire	907
Duffy, Christopher	312	Evans, Christopher H.	891	Finan, Katherine M.	952
Dufour, Jason	340	Evans, Claire F.	145	Finegold, Milton	159, 174, 873
Dunbar, Cynthia	705, 707, 708	Evans, Jeremy	1027	Finger, Carsten	740
Dunn, Marilyn	918	Evans, Tom	486	Fink, David	213, 214, 219, 221
Dupré, Loïc	906	Evans-Galea, Marguerite	179	Fink, Tamara L.	501, 505, 849
Dussel, Maya Otto	696	Everett, Ruth	862, 1012	Finke, Stefan	18
Dwyer, Roisin M.	29	Everts, Maaike	21	Finn, Jonathan D.	314
Dylla, Douglas E.	491, 688	Ewerling, Sonja	660	Finn, Ronald	258, 983
Ebeling, Saskia B.	331	Excoffon, Katherine J. D. A.	768	Finn, David J.	220
Eberle, Peter	384	Ezell, Edward L.	477	Fioretti, Daniela	149
Ebert, Oliver	1035	Ezquer, Fernando E.	183	Firm, Christina S.	688
Echeagaray, Patricia	1003	Fabre, Sylvie	457	Fischer, Alain	706
Edbrooke, Mark	507	Facchini, Giulia	403, 992	Fischer, Anne C.	489, 493
Edelstein, Michael L.	981	Fachinger, Patrick	512	Fischer, Yvonne	977
Edelstein, Richard M.	981	Fairbairn, Leslie J.	76	Fishbein, Ilia	125, 668
Eder, Matthias	633	Fall, Brent	241, 242	Fisher, Donald	426
Edmondson, Andrew C.	307	Fan, Sheung-tat	625	Fiszman, Marc Y.	924
Edmonson, Shyrie	37	Fan, Xiaohu	861	Fittipaldi, Antonio	365
Eefting, Daniel	203	Fan, Yong	79, 652	Fitzgerald, Julie	719
Egermann, Marcus	891	Fang, Juan	920	Flanigan, Ryan	164
Ehrhardt, Anja	58, 172, 811, 1040	Fang, Yujiang	247	Fletcher, Bradley S.	470
Einfeld, David A.	1020, 1025	Farassati, Faris	62	Fliesler, Steven J.	231
Einhorn, Thomas A.	891	Farese, Ann M.	915	Florer, Jane B.	381
Einsele, Hermann	355, 562	Farinati, Fabio	597	Flotte, Terence R.	98, 101, 106, 169, 377,
Ekker, Stephen C.	154	Farley, Raymond	507		436, 493, 530, 1055
Ekmekcioglu, Suhendan	955, 1008	Farlow, Samuel J.	430	Flotte, Terry R.	875
Eferink, Marieke GL	28	Farmen, Sara L.	110	Flygare, Johan	900
Eliason, Steven L.	717	Faro, Carlos	842	Fodor, Istvan	807
Eliopoulos, Nicoletta	911, 918	Farson, Deborah	82, 290, 775	Fodor, Nadja	807
Ellard, Fiona	235, 1062	Faulkner, Eric	1054	Fojo, Tito	618
Ellefson, Barry L.	145	Faulkner, John	242	Follenzi, Antonia	631, 906
Ellinwood, N. Matthew	855	Faust, Susan M.	19, 91	Follenzi, Antonio	661
Ellis, James	402	Fautsch, Michael P.	335	Fong, Timothy C.	635
Elouahabi, Abdelatif	478	Fauvel-Lafeve, Francoise	285	Fontaine, Francois	918
Ely, Abdullah	553	Fazio, Vito Michele	149	Fontes, Michel	805
El-Zawahry, Ahmed M.	676	Federico, Maurizio	375	Forbes, Stuart	166
Embry, Jeniffer E.	874	Federoff, Howard J.	43, 211, 222, 226, 552	Formisano, Pietro	647
Embry, Jennifer E.	878, 879	Federspiel, Mark J.	77, 613	Forrest, M. Laird	361
		Fedorova, Elena V.	733	Forsayeth, John R.	215, 233, 530

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Forshaw, Michael	23, 741	Gage, Fred H.	44, 525	Gerritsen, Winald R.	59, 121, 288, 289,
Fortes, Puri	61, 270, 648	Gaggar, Anuj	136, 276	Gershon, Jill	463, 594
Fortin, Anouk S.	927	Gaidosh, Gabriel S.	251	Gerson, Stanton	156, 808
Fouquet, Françoise	225	Galasso, Gennaro	386	Geurts, Jennifer L.	914, 1006
Foust, Kevin D.	1055	Galipeau, Jacques	349, 911, 918, 972	Gharabeh, Burhan	823, 1042
Fowler, Daniel H.	950	Gallagher, Patrick G.	902	Ghazizadeh, Soosan	245, 246, 930
Fradette, Julie	164	Gallaher, Sean D.	773	Ghosh, Debadyuti	657
Fraefel, Cornel	277	Galldiks, Norbert	995	Giacca, Mauro	131
Fraites, Jr, Thomas J.	250	Galun, Ethan	921	Giannaris, E. Lela	16, 197, 236, 365, 600
Francine, Dugré	1005	Gambhir, Sanjiv S.	975	Gibson, Ron L.	432, 1060
Francis, Jennelle	284, 296, 608, 979	Gambotto, Andrea	383, 554, 778, 896	Giddings, Angela M.	508
Franco, Luis M.	339, 699, 867, 1054	Gamradt, Seth	887	Giesbrecht, Cory	81, 492, 497, 998
Frandsen, Joel	165, 963	Gang, Wu	468	Gil, Jose S.	679
Frandsen, Joel L.	279, 823, 1042	Gangadharan, Bagirath	253, 399, 912	Gilbert, James R.	773
Frank, Matthew G.	530	Gangenahalli, Gurudutta U.	651	Gilbert, Renald	334
Frank, Michael M.	1012	Gansbacher, Bernd	573	Gilbert, Richard A.	70, 774
Frank, Robert N.	1064	Gansemier, Nicholas	768	Gilboa, Eli	822
Fransden, Joel L.	871	Ganser, Arnold	633	Gill, Deborah	588
Fränzel-Luiten, Erna	1046	Gao, Guangping	88, 90, 338, 341, 373,	Gill, Deborah R.	496
Fraser, Nigel	217, 223		568, 569, 692, 1016	Gillespie, G. Yancey	143, 144, 465, 494
Fratantoni, Joseph C.	198, 583	Gao, Guang-ping	1048	Gillies, Stephen D.	1031
Frater, Arlene J.	391	Gao, L.	499	Ginn, Samantha L.	124, 267
Fredrickson, Craig T.	737	Gao, Wentao	554, 778	Ginsburg, Daniel S.	405
Freund, Christian T. F.	785	Gao, Xiang	812	Giordano, Frank	814
Freytag, Svend O.	1033, 1034	Gao, Yongxin	939, 1000	Giordano, Frank A.	331
Friedmann, Theodore	400	Garcia, Felix	173	Giordano, Frank J.	916
Frilling, Andreja	943	Garcia-Sastre, Adolfo	1035	Giorgio, Todd D.	931, 936, 1049
Friz, Jennifer L.	999	Gardmann, Thomas	429	Giovannangeli, Carine	27
Fromes, Yves Y.	924	Gardner, Thomas A.	604, 966, 1021	Giroglou, Tsanan	471
Frosst, Phyllis D.	461	Garnier, Alain	537	Giuliano, Rita	977
Fruehauf, Stefan	331, 344, 916	Garrigue, Alexandrine	706	Glader, Bertil	222
Frykholm, Karolin	663	Garrison, Brian A.	817	Glasgow, Joel N.	1002
Fu, Haiyan	94, 860, 864	Garrison, Brian S.	815	Glenn, Pierce F.	122, 129, 662, 664
Fu, Kailing	69	Garrity-Moses, Mary	45, 532, 735	Glick, Adam	102
Fu, Shuang	990	Gasc, Jean-Marie	967	Glimm, Hanno	859
Fuchs, Renate	835	Gasiorowski, Joshua Z.	812	Glockzin, Gabriel	345, 706
Fuerer, Christophe	616	Gasmi, Mehdi	227, 397, 527, 536, 539	Glorioso, Joseph	440
Fuess, Sally	13, 337	Gavrikova, Tatyana	788, 971	63, 164, 213, 214, 219,	
Fujiki, Minoru	650	Gazdar, Amiq	512, 925	220, 221, 653, 794, 806	
Fujimiya, Mineko	39	Gazit, Dan	384, 827	Glover, Colin	636
Fujimoto, Kenichi	49	Gazit, Zulma	827	Go, Wentao	383
Fujino, Masayuki	656	Ge, Rongbin	150	Godwin, Simon	756, 760
Fujioka, Yoshio	726	Ge, Shundi	696	Goerlitz, Nico	18
Fujisawa, Takehiko	571	Ge, Ying	290	Gofrit, Ofer	30
Fujita, Tetsuo	291, 356	Gearhart, Brian	885	Goin, William F.	63
Fukunaga, Yoshitaka	865	Ged, Cecile	701	Goins, William	214
Fukushima, Kazuhito	604	Geddes, Duncan	473, 480, 507, 720, 935	Goins, William C.	219
Fukushima, Takashi	272	Gedeon, Christopher R.	501, 505, 849	Goins, William F.	164, 221, 794
Funeshima, Naoko	656	Gehlbach, Peter	57	Gojkovic, Zoran	591
Fung, Peter W.	625	Geiser, Thomas	512	Gokhale, Prafulla	617
Furlan, Roberto	46	Gelovani Tjuvajev, Juri	258, 983	Goldberg, Iris	969
Fussenegger, Martin	715	Gendler, Sandra J.	29	Goldman, Steven A.	224
Futin, Nicolas	119	Genise, Hilary L.	1056	Goldnau, Daniela	18
Fux-Cametti, Catherine	170, 409	Genoni, Michele	925	Goldsmith, C. M.	1029
Gabriel, S. E.	687	Gentile, Alessandra	2	Goldsmith, Corinne M.	769
Gabrielson, Nathan	361	Georgiadis, Tassos	36	Goldsmith, Merrill E.	618
Gackowski, Judith	667	Gerard, Christopher J.	760	Goldstein, Harris	370
Gaensbacher, Bernd	440, 612	Gerloni, Mara	564, 1043		125

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Gollapalli, Santhi J.	618	Griffith, Linda G.	850	Håkansson, Emma	825
Goltsov, Alexei A.	291	Griffith, Thomas S.	944	Hakkarainen, Tanja	442, 601, 602, 739
Gomez Vargas, Andrew	35	Grimm, Dirk	96, 371	Halbert, Christine L	508
Gommans, Willemijn M.	293	Grimm, Elizabeth A.	955, 1008	Haldrup, Christa	734
Gonçalves, Christine	682, 835	Griscelli, Franck	967	Hall, Brett M.	973
Goncharov, Tanya	969	Grist, John	208	Hall, Leon L.	680
Goncz, Kaarin K.	691	Grizzle, William E.	21	Hallak, Louay K.	1024
Gonzalez, Hugo	820	Grompe, Markus	13, 826, 873	Hallden, G.	274
Gonzalez-Aseguinolaza, Gloria	319	Groothuis, Geny M. M.	28	Hallden, Gunnar	284, 296, 608, 979
Gonzalez-Edick, Melissa A.	1058	Gross, Christine D.	590	Hallek, Michael	18, 95
Gonzalez-Murillo, Africa	916	Gross, Gerhard	384	Hamada, Hirofumi	47, 189, 190, 771,
Good, Margaret	10	Grossman, William	192	926, 933, 938	
Goodrich, Laurie R.	892	Grosso, Anka	612	Hamada, Hiromi	343
Gopal, Vijaya	318, 482	Grieffin, Beverly E.	450	Hamada, Katsuyuki	989
Gopalan, Began	954	Gruber, Tanja A.	574	Hamaguchi, Isao	900
Gorbatyuk, Marina	654	Gruenert, Dieter C.	316	Hamanaka, Sanae	330
Gorbatyuk, Oleg	420	Grunda, Jessica M.	664	Hamann, Andreas	468
Gorman, Gregory S.	322	Gruneich, Jeffrey	498	Hamilton, Melissa	57
Gorman, Joe	937	Grzybowski, Bradley N.	730	Hamman, Kelly	873
Gorman, Robert C.	937	Gu, Jianren	839	Hampl, Juergen A.	277
Gorvel, Jean-Pierre	835	Gu, Jinfa	448	Han, Jay	718
Goss, James R.	214, 220, 221	Gu, Shuo	411	Han, Ziquin	266
Gotoh, Akinobu	598, 604	Guan, Bo	309	Hanai, Koji	830
Gough, Michael	23, 256, 352	Guan, Min	576	Hanawa, Hideki	5, 707
Gouma, Dirk J.	130	Guenechea, Guillermo	916	Hanazawa, Kisaburo	910
Goverdhana, Shyam	790	Guenther, Margaretha	159, 894	Hanazonno, Yutaka	703
Graat, Harm C. A.	289	Guffey, M. Brad	1031	Hancock, Rick	312
Grabowski, Gregory A.	854	Gugger, Mathias	512, 925	Hang, Shaohua	220
Graepler, Florian	590	Guggino, William B.	489, 493	Hankins, Gerald R.	889, 890
Graf, Hansjoerg	590	Gui, Tong	31	Hanks, Brent A.	347
Graham, Delyth	127, 391, 666	Guido, Maria	597	Hannaman, Drew W.	145
Grande, Alexis	403	Guidotti, Luca	1019	Hannun, Yusuf A.	676
Granelli-Piperno, Angela	124	Guillonneau, Carole	329	Hans, Volkmar	977
Grant, Rebecca L.	218, 341, 1048	Gunnel	450	Hanss, Basil	733
Gray, John	707	Gunther, Roland	871	Hantraye, Philippe	1062
Greco, Olga	962	Guo, Yan	839	Hao, Junshan	536
Green, Anthony P.	426, 793	Guo, Zongsheng	65	Hao, Zhengrong	936
Greenberg, Philip	350	Gupta, Anurag	1064	Harada, Akiko	964
Greenberger, Joel S.	513, 671, 673	Gupta, Pallavi	651	Harada, Yoshinori	942
Greenberger, Shoshana	969	Guschin, Dmitry	302, 324, 931	Harats, Dror	969
Greengold, Diana S.	790	Gusella, G. Luca	733	Harding, Cary O.	873
Gregor, Michael	9, 590	Guy, John	212, 437	Harding, Thomas	775, 974, 1058
Gregorevic, Paul	243, 433, 718	Guy, Louis-Georges	927	Hardy, Sarah	393
Gregory, Lisa G.	36, 235	Haard, Hans J. W.	423	Hargrove, Phillip W.	5
Gregory, Philip	233, 302, 324, 325, 326, 531, 628, 649, 713, 901, 931	Haas, Andrew	572	Harkey, Michael A.	299
Greiner, Suzanne	306	Haas, Dennis L.	8	Harkins, Richard N.	238, 791
Greiner, Suzanne M.	281, 1024	Hacein-Bey-Abina, Salima	706	Harmsen, Martin C.	293, 467
Grenet, Christophe	285	Hacker, Christian	846	Harn, Horng Jyh	283
Grenman, Reidar	118	Hackett, Neil	196, 338, 372, 488, 558, 567, 570, 689, 1015, 1060	Harper, Scott Q.	209, 717
Grez, Manuel	908	Hackett, Perry	165, 823, 824, 871, 1042	Harrington, Kevin	352
Gridley, Daila S.	807	Hackett, Neil R.	432	Harris, David	195
Grieger, Joshua C.	17	Hadac, Elizabeth M.	1026	Harris, David T.	263
Griesenbach, Uta	473, 480, 495, 496, 507, 721, 935, 720	Hagenbeek, Anton	331	Harris, Reuben S.	87
Griesmann, Guy E.	77	Hagner, Nicole	646	Harrison, Lynn	605
Griffey, M.	42	Haisma, Hidde J.	28, 130, 153, 293, 665, 716	Hartigan-O'Connor, Dennis	240, 460
Griffin, Beverly E.	608	Hakakha, Amir	79	Hartl, Irene	614
				Hartung, Markus	803

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

2004

### Abstract Author Index

Harvey, Ben-Gary	488	Henning, Amy E.	973	Hodge, Sandra J.	952
Harvey, Mary	24, 281, 327	Henning, Petra	663	Hodges, Joanna	555
Hasegawa, Mamoru	189, 495, 496, 703, 720, 721, 722, 723, 933	Hensley, Scott E.	719	Hodgson, Clague	74, 818
Hasegawa, Yuichi	272	Herchenroeder, Ottmar	336	Hodish, Israel	969
Hashiguchi, Takao	582, 910	Hermans, Pim	423	Hoeben, Rob	114, 132, 134, 261, 286, 663
Hashimoto, Hideo	1036	Hermens, Wim T.	40	Hoegset, Anders	669
Hashimoto, Masahiko	1015	Hermens, Wim T. J. M. C.	423	Hoeller, Daniela	797, 987
Haskins, Mark	162, 855, 857	Hermiston, Terry W.	238	Hofer, Eva K.	227
Hassani, Zahra	471	Hermonar, Paul L.	257	Hofer, Katrin	527
Hassid, Aviv	956	Hermonat, Paul L.	431, 587, 762, 799, 800	Hoff III, Henry	813
Hasz, Diane	360, 963	Hernandez, Juan	918	Hoffman, Allan S.	639
Hatano, Norikazu	175	Hernandez-Alcoceba, Ruben	580, 978, 980	Hoffman, Andrea	384
Hattan, Naoichiro	738	Herrbrich, Phillip	259	Hofmann, Andreas	660, 997
Hauchecorne, Michelle	504	Herrbrich, Phillip	606	Hofmann, Franz	1057
Hauck, Bernd	747	Herrbrich, Phillip E.	898	Holder, Maxine V.	36, 166, 235
Haug, Stefanie	435	Herrmann, Isabell	623	Hollis, Roger P.	416, 1037
Haurigot, Virginia	173	Hersh, Louis B.	525	Holm, Per Sonne	440, 612
Hauschka, Stephen D.	180	Herzer, Sibylle U.	138	Holman, David H.	676
Hauschka, Steven D.	242	Herzog, Christopher D.	227	Holmes, Jonathan	10
Hauschulz, Kai	742	Herzog, Roland	185, 1002	Holmes, Mark D.	952
Hauswirth, William W.	212, 654	Herzog, Roland W.	33, 103	Holmes, Michael	713, 714
Havenga, Menzo J. E.	114	Hess, David	606	Holmuhamedov, Ekhson L.	392
Hawley, Robert G.	186	Hess, David A.	898	Holterman, Mark J.	116
Hawley, Teresa S.	186	Hewes, Becker	253	Holtzclaw, David	912
Hawthorne, Wayne J.	405	Heyes, James	679	Holz, Eric	1064
Hay, John G.	278, 292, 690	Hickey, Melissa A.	171	Homicsko, Krisztian Gy	616
Hayakawa, Takao	24, 113	Hickey, Reed	491, 688	Homma, Junpei	254
Hayden, Michael R.	40	Hidaka, Chisa	931, 1049	Hong, Bum Kee	707
Hayek, Alberto	400	High, Katherine	888, 892	Hong, Chang-Sook	221
Hazboun, Tawfiq	793	High, Katherine A.	1002	Hong, Q. Y.	499
He, Cheng-Yi	141		32, 37, 103, 104,	Hong, Saw See	663
He, Lin	102		307, 345, 1047	Hong, Seung-Hee	615
He, Tongrong	199, 392	Hilken, Gero	943	Hong, Young Bin	863
He, Xianghui	263	Hill, Richard	296, 979	Hong, Youngtae	396
He, Xiaohua	209, 717	Hillen, Wolfgang	978	Honma, Kimi	304, 830
He, Y.	521	Hillery, Elizabeth	480	Honma, Yukiko	189, 190, 926, 933, 938
He, Ying	282	Hillary, Liz	496	Honmou, Osamu	47
He, Yulong	974	Hinata, Nobuyuki	598, 604	Hood, Jill	1009
Heagerty, Anthony M.	204, 395	Hinch, Linda	107, 931	Hoong, Nien	994
Heale, Bret	548	Hinton, Andrew	400	Hoots, Keith	1002
Hebbel, Robert P.	200	Hirai, Hideyo	262	Hormann, Robert E.	999
Hebel, Henry	820	Hirai, Sachie	933	Hörnsten, Per	766
Hecker, James G.	681	Hirao, Yoshihiko	845	Horsburgh, Brian	627
Heggestad, Arne	470	Hiratsuka, Masaharu	996	Hortelano, Gonzalo	35, 167
Heguy, Adriana	488	Hironaka, Takashi	723	Horwitz, Mitchell E.	915
Heinkelstein, Martin	336	Hiroyama, Takashi	910	Hoshiya, Hideyoshi	991
Heiss, Wolf D.	25, 995	Hirsch, Raphael	457	Hosper, Geke A. P.	153
Heitner, Sheila	292	Hirsch, Tobias	787	Hotz-Wagenblatt, Agnes	916
Helder, Marco N.	289	Hirschberger, Johannes	573	Hou, Dongming	934
Heller, Glenn	555	Hisa, Yasuo	348, 584	Houkin, Kiyohiro	47
Heller, Loree C.	822	Hisayasu, Sanae	311, 866	Howell, John McC	244
Heller, Richard	822, 948	Hishikawa, Tomohito	205	Howell, Kyle	10
Helms, Gregory A.	827, 889, 890	Hiti, A.	702	Hricisak, Lauren	513
Hematti, Peiman	705, 707	Hobart, Peter	486	Hsu, Catherine	461
Hemminki, Akseli	442, 960, 970	Hobbs, Lea Ann	89	Hsu, Chi	541, 993
Hengge, Ulrich R.	953	Hochberg, Abraham	30, 280	Hsu, Chih-Chien	550
		Hodge, Greg	952	Hsu, Hui-Chen	640, 644, 1047

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Hsu, Keng-Fu	595	Ide, Lucienne	912	Jaime, Jairo	135
Hu, Huimin	234	Ideno, Junichi	187	Jamieson, Andrew C.	309, 531, 649, 659, 714
Hu, J.	499	Iggo, Richard	616	Janelsins, Michelle C.	226
Hu, Jennifer	274	Igonkin, Dmitri	543	Janet, Forstner F.	685
Hu, Jim	110, 685	Iida, Akihiro	721, 722, 723	Janik, John E.	456, 575
Hu, Jingjiong	706	Iizuka, Sayoko	858	Janik, Przemyslaw	201
Hu, Yun	65	Ikawa, Masahito	994	Janson, Anneke A. M.	638
Huang, Bijun	561	Ikeda, Uichi	388	Jarajapu, J. P.	941
Huang, Bin	524	Ikegami, Akiko	629	Jaroszeski, Mark J.	822
Huang, Jialin	561, 783	Ikeguchi, Kunihiko	49, 1051	Jarrahan, Sohail	810
Huang, Jinghua	189, 190, 926, 933, 938	Ikehara, Susumu	703	Jarraya, Bechir	1062
Huang, Jing-Hua	946	Ikemoto, Madoka	378	Jaspar, Jean-Marie	641
Huang, Katie	112	Illes, Marc A.	477	Jassar, Arminder S.	572
Huang, Leaf	146, 509	Im, Dong-Soo	951	Jauernig, Jurgen	511
Huang, Shaohua	214, 219, 806	Imagawa, Tomoyuki	457	Jean-Pierre, Bouchard	1005
Huang, Shao-Ling	483	Imanishi, Jiro	262, 348, 584, 942	Jeffes, Edward W. B.	585
Huang, Wenlin	561, 780, 783	Imbert, Anne-Marie	805	Jeffs, Lloyd B.	679
Huang, Yan	936, 1049	Imbert, Jean	54	Jenkins, Connie	619
Huang, Yao Qi	278, 292, 690	Ingesson, M.	796	Jenkins, Denison D.	308
Huang, Yong	817, 819	Ingrassia, Angela	698	Jennelle	450
Huang, Yu	192	Innis, Laura	397	Jennings, Jerilyn S.	94, 860, 864
Huang, Zan	58, 371, 1039, 1040	Ino, Yasushi	804	Jennings, Lisa K.	956
Huard, Johnhny	246	Inoue, Hiroyuki	629	Jensen, Michael C.	354, 369
Huard, Johnny	245, 385, 632, 885, 895, 930	Inoue, Makoto	189, 721, 723, 933	Jensen, Ryan L.	346, 744
Huey, Joeseph	438	Inoue, Naokazu	113	Jeong, Jin-Sook	615
Humblet-Baron, Stephanie	904	Inoue, Ryo	650	Ji, Xiaorong	291, 356
Humeau, Laurent	1003	Ireton, Greg C.	1030	Jia, Zhen	1041
Hummel, Horst D.	355	Irwin, Michael	635	Jian, Weiguo	785
Humperf, Per M.	468	Isayeva, Tatyana	434	Jiang, Canping	794
Humphries, R. Keith	704	Iseki, Tohru	672	Jiang, Haixiang	1012
Hung, Chien-fu	268	Ishibashi, Shun	187	Jiang, Haiyan	103, 753
Hung, Mien-Chie	295	Ishida, Akihiko	517	Jiang, Jiangang	428
Hunt, Kelly	675	Ishii, Hajime	910	Jiang, Jianghong	347
Hurtado, Andres	519	Ishii, Keiji	190	Jiang, Xuan	357
Hurwitz, Mary Y.	133	Ishii, Michiaki	942	Jiang, Xuesong	357
Hurwitz, Richard L.	133	Ismail, Mourad	914	Jiang, Zhilong	248
Huston, James S.	267	Israel, Yedy	183	Jimenez, Daniel F.	984, 988
Hutchins, Beth M.	897	Itah, Refael	801	Jimenez-O., Alejandro	320
Hutchison, Sylvia	1002	Ito, Hiroshi	304	Jin, Da-Yun	31
Huynh, Bernard	252	Ito, Isao	500, 954	Jin, Fang	238
Hwang, Jennifer	227	Ito, Tomoko	840, 847	Jin, Xiangyang	177, 905
Hwang, Kyung-Sun	951	Ito, Yoshinori	47, 189, 190, 926, 933, 938	Jin, Zhengmin	415
Hyatt, Susannah L.	501, 505	Iurescia, Sandra	149	Johannisson, Jenny	663
Hyde, Stephen C.	143, 144, 465	Iwai, Masaki	942	John, Justin	265
Hyde, Steve	507	Iwamoto, Aikichi	73	Johns, Rachel E.	639
Hyde, Steve C.	494, 495, 721	Iwata-Okada, Mayumi	758	Johnson, Betty H.	477
Hyde, Steven	512, 925	Izawa, Kiyoko	672, 910	Johnson, Bryan D.	920
Hyman, B. T.	796	Izumi, Miru	122	Johnson, Douglas H.	335
Hyndman, Laura	479	Jabbour, Nadine	70	Johnson, Kirk W.	102
Iacampo, Sandra	927	Jackson, David W.	850	Johnson, Larry G.	503
Iaccarino, Guido	386	Jacobs, Andreas H.	25, 277, 803, 995	Johnson, Mai	975
Iannitti, David	174	Jacobsen, Frank	787	Johnson, Philip R.	346, 744
Ibad, Raoul Torero	475	Jacobson, Lindsey	889, 890	Johnson, R. P.	546
Ichikawa, Tomotsugu	47, 205	Jacobson, Samuel G.	218	Johnston, Patrick	62
Ichimura, Keiichi	1061	Jadot, Michel	140	Johnstone, Brian	659, 934
Ichinose, Hiroshi	1051	Jadus, Martin R.	585, 635	Joho, Shuji	192
Idamakanti, Neeraja	445	Jahroudi, Reza	585	Joiner, Michael C.	962

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Jolly, Douglas	83	Kang, Yubin	4, 171	Kennedy, Bob	138
Jones, Michele	245, 246	Kao, Chinghai	604, 966, 1021	Kepes, Steven	5
Jones, Steven	373	Kao, Pai C.	306	Keravala, Annahita	809
Jong, Berry de	872	Kaplitt, Michael G.	751	Kessels, Helmut W.	269
Joo, Chang Wan	407	Kapoor, Veena	572	Kessler, Barbara	660, 997
Joo, Chul-Hyen	757	Karim, Mahzuz	352	Kessler, Paul	1064
Jooss, Karin	974, 1058	Karlsson, Stefan	900	Keswani, Sundeep G.	56
Jooss, Karin U.	578	Karolewski, Brian A.	1063	Ketchum, Eli	539
Jordan, Heather	568	Karp, Phil	760	Kethchum, Eli	536
Jorgensen, Michael	321, 487	Karp, Philip H.	110	Ketola, Anna	739
Jouvenot, Yann	233, 325, 649	Kasahara, Nori	779	Key, Nigel	165
Judge, Luke M.	237	Kasahara, Noriyuki	742	Keyser, Kirsten A.	1
Juerchott, Karsten	440	Kaslow, David C.	486	Khakhouline, Tatiana	409
Jun, Liu	213	Kaspar, Brian	20	Khalaghizadeh, Sakineh	585, 635
June, Carl H.	1003	Kaspar, Brian K.	44	Khan, Amir S.	139, 1044
Jung, Cho-Rok	951	Kasper, Michael	468	Khare, Pranay D.	613
Jung, Cindy	730	Kaspers, Gertjan J. L.	289	Khazi, Fayaz R.	307, 345
Jung, Haeng-Im	615	Kastelein, John J. P.	40	Khomenko, Tetyana	585
Jung, Sung-Chul	181, 863	Kasturi, Sudhir P.	838	Kiang, Anne	869, 1012
Juretzek, Thomas	336	Kasuya, Kazuhiko	372	Kida, Miyuki	175
Jurgens, Lisa	808	Katakam, Anand K.	813	Kieda, Claudine	836
Kabanov, Alexander V.	852	Katayama, Yasuo	388	Kiefer, Thomas	900
Kachapati, Kritika	71	Katherine, Ponder	695	Kiem, Hans-Peter	1, 301, 412, 704, 913
Kadmon, Dov	356	Katoh, Motonobu	642, 991, 996	Kierlin, Monique N.	903
Kaess, Bernard	95	Katsura, Ken-ichiro	388	Kierstead, Timothy D.	426, 793
Kaestle, Christine	277	Katusic, Zvonimir S.	199, 392	Kiewiet, Nathan	717
Kafri, Tal	8, 69, 75	Katz, Anna B.	56	Kikuchi, Kan	394
Kaiser, Larry R.	572	Kawabata, Ken	865	Kikuchi, Toshiaki	117
Kakeda, Minoru	996	Kawabata, Kenji	113	Kilpatrick, Michael W.	381
Kakehashi, Akihiro	187	Kawakami, Masanobu	187	Kim, Chang-Min	615
Kakinohana, Osamu	535	Kawakami, Yosuke	116, 122, 129, 620,	Kim, Eun Young	863
Kaleko, Michael	67, 728		664, 964	Kim, Eunhee	441, 444
Kaliberov, Sergey A.	674	Kawakami, Yutaka	1028	Kim, Hong Sung	472
Kaliberova, Lioudmila	629	Kawamura, Nobuaki	175	Kim, Hyejoo	645
Kaliberova, Lyudmila N.	674	Kawano, Takahito	831, 847	Kim, In-Hoo	615
Kalinke, Ulrich	560	Kawasaki, Katsuyoshi	49	Kim, Jae Ho	1034
Kalish, Richard S.	657	Kawase, Takeshi	287	Kim, Jae Woo	453
Kallman, Lisa	619	Kay, Mark	1002	Kim, Jaesung	946
Kamalati, T.	274	Kay, Mark A.	13, 58, 96, 105, 141, 148,	Kim, James	367, 548
Kaman, Wendy E.	638		172, 308, 337, 371, 811, 815,	Kim, James D.	366, 411
Kambara, Hirokazu	624		817, 819, 826, 876, 1039, 1040	Kim, Ji-seon	364, 841
Kamidono, Sadao	598, 604	Kaye, Robin	1002	Kim, John	358
Kaminsky, Stephen M.	432	Kazazian, Jr, Haig H.	779	Kim, Joo-Hang	84, 441, 444, 581, 945
Kan, Yuet Wai	192	Ke, Weng	712	Kim, Joonggon	396
Kanai, Ryuichi	287	Kean, Tara	402	Kim, Keun Sik	472
Kaneda, Yasufumi	228, 297, 517	Kearns, Sean	851	Kim, Mi-Dong	407
Kaneda, Yasuhumi	191	Keefer, Edward W.	737	Kim, Pyung Hwan	946
Kaneko, Shin	272, 330	Keinan-Adamsky, Keren	384	Kim, Pyung-Hwan	945
Kaner, Robert J.	543	Keirstead, Hans	232	Kim, Soon Sun	453
Kanerva, Anna	442, 960	Kelkar, Samir	755	Kim, Sujeong	396, 407
Kang, Bong-Su	821	Keller, Gordon	922	Kim, Sung Jin	757
Kang, Dae-Bong	444	Keller, Michael	321, 487	Kim, Sung Wan	193
Kang, Elizabeth	915	Kelley, Mark R.	282	Kim, Sunyoung	396, 407
Kang, Hong-Yo	884	Kemball-Cook, Geoff	166	Kim, Taewoo	268
Kang, Lu	864	Kemball-Cook, Geoffrey	36	Kim, Yoo-Kyun	757
Kang, Seock	407	Kempf, Tina	573	Kim-Park, SangAe	21
Kang, Yoon A.	441	Kendrick, Kerri A.	497	Kimura, En	241, 242, 379

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Kimura, Hiromitsu	406	Kohlbrenner, Erik	420	Kruger, Warren	181
Kinch, Michael S.	976	Kohn, Donald B.	8, 177, 408, 416, 545, 574, 693, 696, 904, 905, 984, 988, 1037	Ksenich, Pamela	1006
Kindy, Mark S.	525	Koike, Hiromi	517, 940, 1036	Kubal, Aarup	449
King, C. Richter	57, 993, 1025, 1020	Kojima, Hideto	39	Kubo, Atsushi	922
Kingsman, Alan J.	208	Kojima, Hiroshi	272	Kubo, Shuji	779
Kingsman, Susan	235	Kok, Robert J.	665	Kuge, Hiroyuki	876
Kingsman, Susan M.	83, 168, 208, 528, 736, 1062	Kolls, Jay K.	514, 605	Kuivenhoven, Jan Albert	40
Kingston, Paul A.	204, 395	Kolsut, Piotr	201	Kullmer, Martin	846
Kinoshita, Hiroyasu	311	Kolykhalov, Alexander A.	105	Kumagai, Izumi	63
Kirby, Patrick J.	814	Komarova, Svetlana	115, 129	Kuman, Mohan	999
Kirby, Tyler O.	970	Komiya, Setsuro	446	Kumar, Mohan	813
Kirn, David	284	Koniar, Brenda	871	Kumar, Mohan B.	313
Kirn, David H.	296, 979	Koniar, Brenda L.	824	Kumar, Prasanna	813
Kishida, Tsunao	262, 348, 584	König-Merediz, Sven A.	646, 816	Kumar, Rajat	651
Kitade, Yukio	949	Konkle, Barbara	1002	Kumar, Sanjay	619, 746, 886
Kitamura, Yoshihiro	73	Koo, Taeyoung	441, 444	Kumar, Valluripalli Vinod	682
Kitay, Brandon M.	518	Koolwijk, Pieter	153	Kumar-Singh, Rajendra	515
Kitazawa, Yusuke	656	Koprivnikar, Kathryn E.	1058	Kume, Akihiro	187, 343, 388, 421, 427, 703, 758, 1061
Kitsos, Chris	507	Kordower, Jeffrey H.	227	Kunis, Mike	931
Kjaergaard, Niels	573	Korokhov, Nikolay	124, 126, 662	Kunne, Cindy	872
Kjeken, Rune	151, 157	Kosai, Ken-ichiro	446	Kuntz, Gabriele	355, 562
Klatzmann, David	459	Kotin, Robert M.	230, 419, 717, 759	Kunugiza, Yasuo	940, 1036
Klein, Markus	995	Kottke, Timothy	23, 352	Kuo, Yu-Hung	751
Klein, Michael	1064	Koty, Zafiro	972	Kuppen, Peter J. K.	280
Klepcyk, Patrick J.	505, 849	Kournioti, Chryssanthi	1035	Kuppuswamy, Mohan	1011, 1018
Klinz, Stephan G.	267	Kouskoff, Valerie	922	Kurimasa, Akihiro	642
Klotz, Michael	765	Kovesdi, Imi	689	Kurita, Ryo	910
Kmiec, Eric B.	305, 310, 315, 317	Kowalczyk, Tomasz H.	501, 505, 849	Kuroda, Ryosuke	632
Knapp, Deborah W.	976	Koyama, Yoshiyuki	840, 847	Kuroki, Masatoshi	187
Knight, Brandon	585	Kozarsky, Karen F.	100	Kurozumi, Kazuhiko	47
Knoeber, Catherine	1030	Kozin, Elliot B.	56	Kusaka, Nobuyuki	291
Knoess, Christof	995	Kozlowski, Mirek	376	Kusano, Kengo	195
Knoll, Thomas	184	Krahn, Martin	805	Kutner, Robert H.	792
Knop, David R.	420	Krajcsi, Peter	447	Kvale, Dag	157
Knot, Harm J.	941	Kral, John G.	658	Kwack, Seung Jun	453
Ko, Derek	445, 775	Kramer, Beat P.	715	Kwan, Sau-Ping	905
Ko, Dong Hyun	441	Kramer, Gabriela	980	Kwok, Kevin	536, 539
Kobayashi, Hidenori	650	Kramer, Maria-Gabriela	978	Kwon, Byung-Su	615
Kobayashi, Hiroshi	696	Krasnykh, Victor	971, 1013	Lachance, Jean-Guy	1005
Kobayashi, Kunihiko	175	Krause, Anja	558, 689	Lachapelle, Guillaume	70
Kobayashi, Ryouji	175	Kren, B. T.	178, 985	Lackner, Andrew	340
Kobayashi, Seiichiro	672	Kren, Betsy T.	216	Laczka Ozvegy, Csilla	908
Kobayashi, Takeshi	933	Krendelchtchikov, Alex	124	Lai, E. C.	521
Kobinger, Gary	558, 732	Kreppel, Florian	336, 667	Lai, Li-Ju	655
Kobinger, Gary P.	368, 373, 490, 568, 1016, 1053	Kress, Dagmar	997	Lai, Mayoi	352
Kobune, Masayoshi	189, 190, 926, 933, 938	Kretschmer, Peter	238	Lai, Zhennan	863
Koc, Omer	1006	Krishna, Delfi	729	Laipis, Philip J.	874, 877, 878, 879
Koch, Walter J.	386	Krishnaney, Ajit	439	Lajmi, Ajay	792
Kochanek, Stefan	336, 524, 580, 667	Krishnaney, Ajit A.	45, 532	Lalani, Alshad S.	974, 1058
Köck, Juliana	646	Krisky, David	214, 806	Lamande, Noel	967
Kodera, Mika	49, 1051	Krisky, David M.	164, 794	Lamikanra, Abigail	736
Koeberl, Dwight D.	339, 699, 867, 1054	Krissansen, Geoffrey W.	625	Lan, Ning	903
Koehler, David R.	110, 685	Kristoffersen, Peter	591	Landazuri, Natalia	78, 399
Koestlin, Roberto	573	Kroeger, Kurt	57	Landers, Mark	539
		Krouse, Allen	705	Langbein, Sigrun	957, 959
		Kruegel, Brian	527	Langer, Noga	921

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Langer, Stephen J.	530	Lee, Seong-Wook	611, 615	Li, Linhong	198, 583
Langfield, Kirsten K.	77	Lee, Seung Hoon	453	Li, Lisa	25, 995
Langford, Catherine P.	1031	Lee, YaLi	531	Li, Ming-Jie	367, 410
Lanson, Nicholas A.	415, 514	Lee, Ya-Li	713	Li, Qiliang	899
Larcher, Thibaut	1050	Lee, Yoon-Jong	615	Li, Sheng	241, 242, 379
Largaespada, David A.	165, 360, 823, 824, 963, 1042	Lee, Young-III	821	Li, Shirley	367
Largaspada, D.	985	Lee, Young-Sook	581	Li, Shulin	589
Larochelle, Andre	705	Lee, Zhenghong	832	Li, Weiming	303, 982
Larsen, Steven H.	303	Leeuw, Bertie H. C. G. M. de	114	Li, Xiao jin	910
Latouche, Jean-Baptiste	555	Lefevre, Pierre	770	Li, Xiaog-gang	1051
Lattanzi, Wanda	383, 896	Lefort, Jean	506	Li, Xiao-Kang	406, 656
Lau, Cara	1032	Legrand, Chantal	285	Li, Yanjun	52, 303, 342, 982
Lau, Gabriel	531	Lehmann, Romy	915	Li, Ying	449
Lauer, Ulrich M.	9, 590, 742	Lehmkuhl, Howard	89	Li, Yuanhao	445, 775
Lauer, Ulrike A.	590	Lehn, Jean-Marie	504	Li, Zong-Yi	22, 1010
Lauffenburger, Douglas A.	850	Leike, Kristin	504	Liang, Hai-Dong	935
Laufs, Stephanie	331, 344, 916	Leinwand, Leslie	18	Liang, Yuxin	107, 659, 931
Lavin, Martin F.	596	Lejeune, Laurence	530	Liang, Zhihui	783
Lavoie, Genevieve	1032	Lemken, Marie-Luise	911	Liao, Kuang Wen	283
Lawandry, Shokry	527	Lemkine, Gregory	590	Liao, Shaoxi	862
Lawler, S. E.	796	Lemmens, Raf	471	Liao, Shiao Xi	869
Lawrence, Lorraine	235	Lemoine, Jerome L.	825	Lichtenstein, Drew L.	447, 1011, 1018
Lawrence, Theodore S.	1022	Lemoine, Nicholas N.	509	Lie-A-Ling, Michael	784
Lawton, Anna E.	143	Lemoine, Nicholas R.	979	Lieber, Andre	22, 123, 260, 271, 276, 899, 1010
Le, Long P.	21, 782, 786, 788, 971	Lemoine, Nick	284	Lieber, Andre M.	136
Le, Tannie	425	Lemoine, Nick R.	450, 608	Lieberman, Jay R.	887
Le, Thanh	528	Leonard, Joshua N.	547	Lievens, Joke	120, 387, 563
Le Coultre, Claude	170	Leong, Kam W.	357, 828	Lim, Weiseng	357
Le Doux, Joseph	399	Leopold, Philip L.	488, 755	Lima, Jose	619
Le Doux, Joseph M.	78, 729, 730	Lerner, Seth P.	785	Limberis, Maria	498, 692
Le Mauff, Brigitte	97	Leske, David	10	Lin, Andy	82
Lebherz, Corinna	128, 218, 341	Lessard, Ruth	1002	Lin, Chung-Ren	637
Lebkowski, Jane	232	Leuse, Leonard	240	Lin, Datien	904
Leclerc, Guy	927	Levade, Thierry	868	Lin, Jian Min	1058
Lecocq, Michèle	140	Levine, Bruce	1003	Lin, Jian-Min	974
LeCouter, Richard	1058	Levivier, Marc	641	Lin, Ken-Kuo	655
Ledebur, Jr, Harry C.	476	Levy, Robert J.	125, 200, 668, 937	Lin, Ming-Wei	637
LeDeist, Francoise	706	Lewin, Alfred S.	212, 437, 529, 654, 875, 877	Lin, Richard	539
Ledford, Julie G.	19	Li, Bihua	617	Lin, Shinn Zong	283
Ledoux, Catherine	119	Li, Binghua	448	Lin, Shinn-Zong	1045
Lee, Amy C. H.	679	Li, C.	694, 853	Lindemann, Dirk	336
Lee, Brendan	462	Li, Chengwen	106	Linden, Michael	990
Lee, Brendan H.	159, 643, 894	Li, Chin-Jin	550	Lindholm, Leif	134, 663
Lee, Chang I.	984, 988	Li, Dayuan	431	Lindman, Kristina	766
Lee, Che-Hsin	595	Li, Guangheng	895	Ling, Yiqun	930
Lee, Gary K.	401	Li, Haitang	366	Lingas, Karen	1006
Lee, Heuiran	757	Li, Han	653	Lingfei, Xu	695
Lee, Hyun Joo	453	Li, Hong	285	Link, Charles J.	538
Lee, Jeong-Eun	821	Li, Hongwei	889, 890, 939, 1000	Linton, Gilda F.	915
Lee, Ji Eun	472	Li, Hui	323	Lippin, Yitzhak	921
Lee, Jin-Sung	181	Li, J.	1029	Lisowski, Leszek	72
Lee, Karim	407	Li, Jane	655	Liu, An-Bang	1045
Lee, Keun Bae	765	Li, Jiansheng	323	Liu, An-Hsiang	889, 890
Lee, Minhyung	193	Li, Jin Zhong	889, 890	Liu, Bianling	917
Lee, Patrick	62	Li, Jinzhong	827	Liu, Binlei	265, 266
Lee, Rhee Da	453	Li, Juan	14, 38, 763, 986	Liu, Chunyan	1019
		Li, Lina	759		

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Liu, Dexi	812	Lorenz, Helga	882	Maelandsmoe, Gunhild	669
Liu, Dijie	833	Losordo, Douglas	195	Maeyama, Yoshihiro	175
Liu, Dinggang	936	Lotti, Francesco	403, 992	Magnusson, Maria	663
Liu, Feng	146	Lotze, Mike	65	Maguire, Albert M.	218
Liu, Gumei	229, 230	Louboutin, Jean-Pierre	161, 1053	Maguire, Katie	317
Liu, Guoqing	40	Low, Walter C.	216	Maguire-Zeiss, Kathleen A.	222
Liu, Hong	556	Lowenstein, Pedro R.	459, 661, 790, 1019	Mah, Cathryn	250
Liu, James	45	Loyter, Abraham	842	Mahan, Sheri D.	1030
Liu, James K.	439, 532, 735	Lozier, Jay N.	464	Mahendra, Gandham	619, 886
Liu, Jian-Hua	750	Lu, Aliping	246	Mahendran, Ratha	264
Liu, Jun	290	Lu, Baogen	443, 1023	Maheshri, Narendra	20
Liu, Li	310, 677	Lu, He	285	Mahller, Yoni H.	627
Liu, Linda N.	198, 583	Lu, Kang	94	Maier, Steven F.	530
Liu, Mingju	247	Lu, Xiaobin	6, 1003	Maina, Njeri	303
Liu, Nancy Q.	887	Lu, Yi	956	Majetich, S.	146
Liu, Pei-Qi	324, 659	Lu, Yuanqing	106, 429	Makhija, Sharmila K.	443, 1023
Liu, Perry	238	Luan, Bo	974, 1058	Malech, Harry L.	176, 915
Liu, Qiang	380	Lucas, Michael A.	245	Malecki, Maciej	201
Liu, Ranyi	561	Lucia, Pattarini	188	Malik, P.	702
Liu, Renbin	798	Luk, Alvin	1002	Malik, Punam	701
Liu, Ta-Chiang	284	Lukason, M. J.	853	Mallam, Joshua N.	133
Liu, Tongyao	102, 753	Lukason, Michael	523	Mandel, Ron	941
Liu, Wen	380	Luke, Jeremy	74	Mandel, Ronald	851
Liu, X.	1029	Luketich, James D.	671	Mandel, Ronald J.	420, 529, 1055
Liu, Xinyuan	448	Luna, Jac	424	Mane, Viraj	643, 789
Liu, Yan-Hui	781	Lundin, Karin E.	150	Mane, Viraj P.	159, 462
Liu, Yanna	675	Luo, Phoebe	195, 263	Manfredsson, Fredric P.	529
Liu, Yemin	362	Luo, Rongcheng	257	Manlapaz, Leo	227, 539
Liu, Yi-Lin	104	Luo, Tianci	67, 728	Manley, Kevin	48
Liu, Yong	257, 431, 587, 762, 799, 800	Luongo, Cindy L.	129	Mann, Linda	234
Liu, Yuhe	758, 1061	Lupo, Rossella	375	Manno, Catherine	1002
Liu, Yuli	162, 695, 856	Lupold, Shawn E.	137, 275	Mano, Miguel	842
Liu, YuLi	857	Lusky, Monika	119	Mansfield, S. Gary	712
Liu, Zhenjun	428	Lustig, Kurt	99, 880	Mantwill, Klaus	440
Lo, Chi-Wen	579	Luther, Thomas	468	Manuszak, Ryan	538
Lobitz, Paul	165, 360, 963	Luthra, Pratibha M.	651	Mao, Hai-Quan	357, 828
Lochrie, Michael	752	Luton, Dominique	504	Mao, Qinwen	209, 717
Lochrie, Michael A.	89	Lux, Kerstin	18	Marangoni, Francesco	906
Loecher, Anne	573	Luxembourg, Alain	145	Marcello, Alessandro	16
Loewen, Nils	10	Luzko, Carolyn	696	March, Keith L.	934
Loewen, Nils A.	335	Lyons, Gray R.	968	March, Michael	1017
Logan, Aaron C.	8, 68	Lyszcowicz, Dorota	376	Marchesini, Sergio	41
Logroscino, Carlo A.	383, 896	Ma, Hong	69, 75	Marelli, Ornella	149
Loi, Roberto	691	Ma, Xiaoming	216	Margalith, Michal	486
Loiler, Scott A.	169	Maatouk, Imad	280	Margaritis, Paris	32
Lombardo, Angelo	631	MacDanold, Robert C.	483	Margison, Geoffrey P.	76
Lonez, Caroline	478	MacDonald, Neil	476	Marie, Frenzke	593
Long, Meredith	903	MacDonald, Robert C.	469	Marilyne, Goulet	1005
Long, Y.	521	MacGregor, Rob Roy	1003	Marina, Dapas	188
Longaker, Michael T.	308	Maciejewska, Teresa	577	Marinov, Anthony D.	457
Lonning, S.	853	MacLachlan, Ian	314, 679	Marion, Patricia L.	371
Looije, Norbert	872	MacLeod, James N.	918	Maris, Michael	413
Looney, D. J.	546	MacVeigh, Michelle	844	Marjanovic, Olivera	397
Looney, David J.	298, 397	MacVittie, Thomas J.	915	Markert, James M.	1031
Lopez, Enrique P.	519	Maden, Malcolm	208	Marklund, Stefan L.	677
Lopez-Fuertes, Luisa	816	Maeda, Sakan	604	Markmann, James F.	1053
Lorena, Zentilin	188	Maelandsmo, Gunhild M.	1014	Markusic, David M.	727

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Marples, Brian	962	McCray, Andrea N.	822, 948	Meulenbroek, Robert A.	1014
Marr, Robert A.	525	McCray, Jr, Paul B.	4, 80, 171, 491, 502, 688	Meuse, Leonard	241, 242, 379, 460, 718
Marsala, Martin	535	McCullers, Jon	179	Mewani, Rajshree	617
Marshall, John	854	McDonald, Amy	394	Meyer, Andy	999
Marshall, Nicola	935	McEachern, Kerry	854	Meyerrose, Todd	606
Martin, Dana B.	881	McGrath, Yvonne	265	Meyerrose, Todd E.	898
Martin, J. Tyler	931	McIvor, R. Scott	165, 824, 963, 7, 279, 823, 871, 1042	Mhashilkar, Abner	126, 662, 675, 955, 1008, 1009
Martin, Molly E.	833	McKay, Tristan R.	81, 492	Mhashilkar, Abner M.	124, 500, 954
Martin, Oudega	519	McKee, Hayley J.	544, 559	Mi, Jie	240, 460
Martin, Tyler	531	McKenzie, Tamra	675	Miao, Carol	413
Martineau, Daniel	918	McLachlan, Gerry	144, 496	Miao, Carol H.	158, 904
Martinez, Laura	61	McLaughlin, Pamela M. J.	293, 467	Miao, Fudan	40
Martinez-F., Francisco	320	McMahon, Beth M.	147	Miccio, Annarita	182, 992
Martino, Ashley T.	101	McMahon, Stephen B.	208	Michalakis, Stylianos	1057
Martino, Gianvito	46	McMillin, Douglas W.	253, 399	Michel, Gilles	414
Martino, Silvana	906	McNamara, Andrew	233	Michel, Maurice S.	957, 959
Martins, Ines	1059	McNamara, Andrew R.	325, 326, 649	Michel, Maurice Stephan	184
Martins, Ines H.	209, 717	McPherson, David D.	483	Michele, Daniel E.	491
Martuza, Robert L.	351, 798	McPherson, Sylvia A.	640, 644	Midoux, Patrick	682, 835, 836
Marty, Michel	909	McTaggart, Ryan	748	Migita, Makoto	865
Marwah, Sonia	136	McVey, Duncan	993	Mignet, Nathalie	475
Mas, Alexandre	155	Mech, Christine A.	728	Mijnsbergen, Yvonne	423
Masliah, Eliezer	525	Medico, Enzo	2	Mikkelsen, Jacob G.	815, 826
Massie, Bernard	70, 135, 774, 1032	Medin, Jeffrey A.	861, 868, 950	Mikkelsen, Jacob Giehm	819
Mastenbroek, Jeroen D. C.	59	Medina, Maria F.	490	Miletic, Hrvoje	277, 977
Mastrangelo, Michael A.	43, 211, 226	Medina-Kauwe, Lali K.	844	Miller, A. Dusty	508
Mata, Marina	213, 214, 219, 220	Meghan, Maguire	844	Miller, Aaron	477
Matern, Dietrich	436, 438	Mehta, Arpesh	527, 539	Miller, Andrew D.	321, 487
Mathevet, Patrice	252	Mehta, Jawahar L.	431, 587, 762, 799, 800	Miller, Andy D.	484
Mathiesen, Iacob	151, 157	Mei, Ya-Fang	766	Miller, Daniel G.	526, 749
Mathis, James M.	621	Meijer, Dirk K. F.	665	Miller, Jeffrey C.	51, 713, 714
Matouk, Imad	30	Melas, Catherine	641	Miller, Laurence J.	1026
Matschinsky, Franz	1053	Melcher, Alan	352	Miller, Todd W.	48
Matsuda, Tsuyoshi	650	Melero, Ignacio	270	Miller, William H.	391, 666
Matsumoto, Kazumasa	785	Mellieon, Francesca	589	Miller, William M.	82
Matsumura, Yoshiaki	845	Menander, Kerstin	1007	Milligan, Erin D.	530
Matsuo, Masafumi	604	Mendel, Matthew C.	659	Mills, Kevin	394
Matsushita, Takashi	388, 421, 758	Menez, Andre	374	Mills, Philippe	239
Mauleon, Itsaso	580	Meng, Jiang Hui	296	Milner, Jeffrey D.	901
Maurisse, Rosalie	316	Meng, Xing Li	858	Milosevic, Dragana	328
Mauro, Giacca	188	Mennesson, Eric	835, 836	Milsom, Michael	76
Mavilio, F.	1004	Merchan, Jaime R.	1024	Mimuro, Jun	343, 427
Mavilio, Fulvio	46, 60, 182, 375, 398, 403	Mercier, George T.	111	Minakuchi, Yoshiko	830
Mazarakis, Nicholas	235, 532, 636	Mercier, Skip	894	Mingozi, Federico	33, 37
Mazarakis, Nicholas D.	48, 208, 528, 735,	Meriläinen, Outi	601	Miniero, R.	1004
	1062	Merkler, Kathleen	822	Mir, Lluis M.	285
Mazda, Osam	262, 584, 942	Merling, Randall	1003	Mirmohammadsadegh, Alireza	953
Mazda, Osamu	348	Merritt, James	1007, 1008, 1009	Mirolo, M.	1004
McCabe, Cillian T.	772	Merritt II, J. Lawrence	108, 438	Misawa, Tkeyuki	965
McCaffrey, Anton P.	58, 371	Merten, Christoph A.	333, 560	Mise, Nikica	943
McCarthy, Douglas M.	11, 94	Mertens, Geertrui	120	Mishra, Swaroop	363
McClintock, Kevin	679	Mes-Masson, Anne-Marie	1032	Miskin, James	1062
McCormack, W. Michael	643	Messer, Anne	48	Miskin, James E.	83
McCormack, Jr, W. Michael	159	Meteyer, Tom	999	Mistry, Ajay	166
McCorquodale, Stephanie	34	Meulenbergs, Janneke J. M.	40, 423	Mistry, Ajay R.	36
McCown, Thomas	69			Mitani, Ko	112, 300
McCown, Thomas J.	50				

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Mitchell, James B.	769	Moressi, Christopher J.	4	Nair, Smita K.	588
Mitchell, Lloyd G.	712	Morgan, Richard A.	186	Naka, Toshio	726
Mitchell, Ngan	1019	Mori, Taisuke	858	Nakagami, Hironobu	297
Mitomo, Katsuhisa	495	Morikawa, Masayuki	190, 933	Nakagawa, Katsumi	468
Mitrani, Eduardo	921	Morimoto, Yoshihisa	927	Nakai, Hiroyuki	13, 96, 337, 371
Mitrophonous, Kyri	636	Morishita, Ryuichi	228, 517, 940, 1036	Nakajima, Satoru	175
Mitrophonous, Kyri A.	736	Morisita, Ryuichi	191	Nakajima, Yoshiyuki	876
Mitrophonous, Kyriacos	36	Morris, John C.	29, 456, 575	Nakakura, Takayo	419
Mitrophonous, Kyriacos A.	83, 168, 235, 1062	Morris, Julie	1	Nakamura, Gerald K.	145
Mitrophonous, Kyriacos A.	528	Morris, Kevin V.	298	Nakamura, Kiminori	47, 189, 190, 926, 933
Mittal, Suresh K.	458, 976	Morrison, Brian J.	456, 575	Nakamura, Takafumi	24, 306, 327, 610, 1026
Mittler, Dominik	787	Moser, Ann	225	Nakanishi, Yoichi	629, 964
Miura, Yoshiaki	949	Moses, Mary	439	Nakano, Hiroshi	348, 584
Miyagishi, Makoto	771, 1028	Mosley, Karen L.	36	Nakano, Imaharu	49, 1051
Miyagoe-Suzuki, Yuko	244, 378	Moss, Bernard	65	Nakano, Kenji	63
Miyahara, Ryo	500	Moullier, Philippe	1050	Nakauchi, Hiromitsu	255, 330
Miyake, Koichi	900	Mountz, John D.	640, 644, 1047	Nakayama, Katsutoshi	160, 670
Miyamae, Takako	457	Moustafa, Mohamed E.	522	Nakazaki, Yukoh	582, 910
Miyano hara, Atsushi	535	Moxley, Michael	426	Nalbantoglu, Josephine	972, 1032
Miyata, Teruo	304	Mueller, Markus	142	Naldini, Luigi	2, 3, 41, 332, 631, 661, 906
Miyoshi, Hiroyuki	672, 1028	Muenzer, Joseph	94, 860, 864	Nam, Young Ran	757
Mizrahi, Aya	30, 280	Mukherjee, Santanu	795	Nambiar, Sandeep	953
Mizuguchi, Hiroyuki	24, 113	Mulder, Nanno H.	153	Nantz, Michael H.	677, 681
Mizukami, Hiroaki	187, 343, 388, 421, 427, 758, 1061	Mullick, Alaka	70	Nara, Yuko	49, 1051
Mizutani, Taka	105	Mumm, John B.	955	Naruishi, Koji	291, 356
Moats, Rex	696	Muñoz, Sergio	155	Narula, Navneet R.	937
Moayeri, Morvarid	186	Munson, Keith	756, 760	Narvaiza, Iñigo	648
Mochizuki, Hideki	865	Munson, Keith L.	489	Nash, Joan	809
Mochizuki, Yasushi	378	Muramatsu, Shin-ichi	49, 1051, 1061	Nash, Kevin	420
Moen, Robert C.	501, 505, 849	Muraro, Sara	350	Navas, Patrick A.	299
Mohammed, Sulma	976	Murofushi, Yohiteru	446	Navon, Gil	384
Mok, Hoyin S.	454	Murray, Peter	179	Nawroth, Peter P.	468
Molema, Grietje	665	Murugesan, Selva	1020	Nazarian, Ara	891
Molina, Rene	6	Murugesan, Selva R.	1025	Neale, Geoffrey	5
Molina, Rene P.	67, 728	Muruve, Daniel A.	455, 745, 767, 1014	Neff, Christina	355, 562
Monaco, A. P.	796	Muschellack, Lia K.	417	Neff, Tobias	22, 412, 704, 913
Monahan, Paul E.	31	Muthuman, Karuppiah	948	Nejime, Tetsuya	535
Monfare, Parisa	995	Muzyczka, Nicholas	420	Nelly, Laura	39
Montane, Joel	155	Myers, Kevin A.	736	Nelson, Douglas R.	267
Montier, Tristan	481	Myers, Rae M.	281	Nemet, Katalin	908
Montier, Tristan Y.	479	Myhre, Susanna	663	Nemunaitis, John	1008
Montini, Eugenio	2, 3, 873	Naash, Muna I.	231	Nemunaitis, John J.	1009
Monfrasio, Fabio	560	Nabekura, Tsukasa	255	Nephew, Kenneth P.	452
Moolla, Naazneen	553	Nagahara, Shunji	830	Nervi, Bruno	259, 606
Moon, Ik-Jae	485	Nagahara, Syunji	304	Nettelbeck, Dirk	442
Mooney, Mark P.	385	Nagai, Yoshiyuki	723	Nettelbeck, Dirk M.	620, 970
Moore, Jill	944	Nagamura, Fumitaka	672	Netto, George	1008
Moore, Peter	138	Nagano, Satoshi	446	Neubert, Wolfgang J.	9
Mootien, Sara	902	Nagasawa, Toshiro	272, 330	Neuenfeldt, James	76
Moran, John V.	779	Nagasawa, Toshirou	255	Neufeld, Elizabeth F.	693
Morcos, Michael	468	Nagashima, Takeyuki	299	Neumann, Harold	977
Moreau, Thomas	54	Nagata, Keiko	703	Neves, Silvia S.	592
Moreau-Gaudry, Francois	701	Nagata, Michio	996	Nevins, Joseph	202
Moreno, Jesus	451, 741	Nagy, Dea	330	Ng, P.	521
Moreno, Sonia	816	Nagy, K. Zsuzsanna	102	Ng, Phil	462
		Naik, Arpana	916	Ng, Philip	133, 39, 110, 159, 163, 174, 789, 869
			65		

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Ng, Phillip	894	Ochs, Hans D.	158, 904	Opolon, Paule	285, 967
Ng, Thian C.	607	O'Connell, Philip J.	405	Oppenheim, Ariella	795, 932
Ng, Thomas	174	O'Connor, Maureen	135	Oranje, Paul P. A.	40, 423
Ngiam, Celina	68	O'Connor, Michael K.	29	Orentas, Rimas	808
Nguyen, Hoc	425	O'Connor, Timothy P.	488	Orentas, Rimas J.	156
Nguyen, Natalie	445	Odaka, Yoshi	621	O'Riordan, Catherine R.	210
Nguyen, Tuan Huy	409, 170	O'Day, Terry L.	382	Orlic, Donald	705
Ni, Shaoheng	22, 136, 260, 276, 1010	O'Donnell, Patricia	855	Orr, Harry T.	717
Nichols, Timothy	103	O'Donnell, Patty	162	Ortiz, Aurora	510, 511
Nichols, Timothy C.	34, 159, 161, 163	O'Donnell, Patty A.	857	O'Shea, Cristin E.	988
Nicklin, Stuart A.	127, 666	Oette, Sharon M.	501, 505, 849	Oshima, Hideki	930
Nicol, Campbell G.	666	Ofosu, Frederick A.	167	Oshima, Mitsuo	642, 991, 996
Nicolazzi, Céline	475	Ogawa, Tomoyuki	205	Osonoi, Makoto	910
Nie, Suhua	513	Ogawara, Kenichi	665	Ossevoort, Martine	261
Niedzinski, Edmund J.	147	Ogihara, Toshio	191, 228, 940	Ostrove, Jeffrey M.	227
Nielsen, Villy	421	Ogura, Tsuyoshi	343, 421, 427	Ostrowski, Lawrence	998
Nienhuis, Arthur W.	5, 179, 707	Oh, Hyun-Jeong	181	Ostrowski, Lawrence E.	497
Nietupski, Jennifer B.	854	Ohana, Patricia	30, 280	Otaegui, Pedro J.	155, 173
Nightingale, Sarah	416, 1037	Ohashi, Kazuo	876	Otsu, Makoto	175, 272, 330
Niidome, Takuro	831, 840, 847	Ohashi, Masaki	949	Otsuki, Akihiro	642
Nikles, Daphne	560	Ohashi, Toya	858	Otten, Gillis R.	556
Nikola, Arsic	188	Ohbayashi, Fumi	300	Otto, Edward	712
Nilsson, Per	825	Ohlfest, John R.	165, 360, 824, 963	Oude-Elferink, Ronald	727
Nishikawa, Tomoyuki	297	Ohmi, Kazuhiro	693	Oudrhiri, Noufissa	504
Nishino, Tamon	1001	Ohnami, Shumpei	949	Oury, Jean-Francois	504
Nistor, Gabriel	232	Ohyanagi, Mitsumasa	726	Overman, Justine J.	680
Niu, YunYun	673	Okabe, Masaru	113	Overmeer, Renée M.	59
Nivsarkar, Megha S.	36, 166	Okada, Kaori	383	Owaki, Toshi	496
Nixon, Alan J.	892	Okada, Takashi	187, 343, 388, 421, 427,	Owaki, Toshiyuki	720
Noblitt, Loren W.	976		758, 1051, 1061	Owens, Geoffrey C.	737
Noh, El	889, 890	Okano, Hideyuki	287, 624	Owens, Roland A.	90
Nolan, Edward M.	145	Okano, Motohiko	175	Ozawa, Keiya	49, 187, 343, 388, 419, 421,
Nolan, Garry P.	712	Okanoue, Takeshi	942	427, 703, 758, 1051, 1061	
Noll, Meenakshi	826	Okita, Chiga	991	Ozelo, Margareth	1002
Nolta, Jan A.	259, 606, 898	O'Konek, Jessica	1033	Ozer, Egon A.	566
Nomoto, Tatsuya	388, 421, 758, 1061	Okuda, Tatsuya	831	Pabo, Carl O.	51, 325, 714
Norikane, Satoko	991	Okuyama, Torayuki	656	Pacak, Christina	859
Nornen, B.	546	Oldenburg, Jill	68	Pacak, Christina A.	251
Norris, James S.	676	Olgiati, Clelia	60	Pack, Daniel W.	361
Norton, Judy	605	Olinga, Peter	28	Page, John G.	322
Nour, May	231	Olivares, Eric C.	152, 810, 814	Pageant, Huandong	607
Noureddini, Sam C.	126	Olsen, John C.	81, 492, 497, 503, 998	Painter, Hazel	494, 507
Novack, Deborah	856	Olson, Darin E.	376, 893	Painter, Richard G.	415, 514
Novelli, Francesco	907	O'Malley, Mark	65	Palli, Subba R.	313, 813, 999
Nukiwa, Toshihiro	117	O'Malley, Thomas	855	Palmatory, Elizabeth	937
Nunez, Marco T.	183	O'Malley, Thomas M.	857	Palmer, Donna	133, 159, 163, 174,
Nwanegbo, Edward	554	O'Malley, Tom	162	789, 894	
Nwanegbo, Edward C.	778	Ono, Fumiko	49	Palmer, Donna J.	869
Nyanguile, Origene	125, 668	Ono, Hidetaka A.	788, 971	Palmer, Glyn D.	891
Nygren, Per-Åke	663	Ono, Shigeki	205	Palmer, Lorne	679
Nyugen, Amy	635	Ono, Yasuhiro	47	Palphi, Stephane	1062
Oakley, Suzanne	718	Onodera, Masafumi	175, 255, 272, 330	Palù, Giorgio	597
Obeid, Lina M.	676	Ooi, Jun	672	Pandey, Kusum	58
Oberley, Larry W.	392	Ookawara, Shigeo	388	Pandori, Mark W.	430, 777
O'Brien, Timothy	109, 772, 776	Oosterhoff, Dinja	288, 594	Panet, Amos	921
Ochiya, Takahiro	304, 830	Oosterhuis, Dorenda	28	Pang, J. J.	654
Ochoa, Laura	319	Ophorst, Olga J.	114	Pantuck, Allan	577

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Papadeas, Sophia	50	Peng, Hairong	385, 632, 885, 895	Poenaru, Livia	870
Papaleo, Pierangelo	896	Peng, Kah-Whye	24, 281, 306, 327,	Poeschla, Eric	10
Papayannopoulou, Thalia	899		610, 1026	Poeschla, Eric M.	335
Parekh-Olmedo, Hetal	310, 317	Peng, Peter D.	172	Poirier, Amy E.	101
Parikh, Nehal S.	294	Peng, Weidan	603	Pola, Enrico	383, 896
Park, C.-W.	985	Pepper, Karen	177, 408, 545, 574	Poleman, James	829
Park, Chang-won	216	Pepper, Karen A.	416	Polley, C. E.	146
Park, Eun-Sook	181	Perabo, Luca	95	Pollock, Roy	647
Park, Frank	334, 415, 738	Perani, Laura	60	Ponder, Bruce A.	943
Park, Hyun	821	Pereboev, Alexander V.	116, 267	Ponder, Katherine	855, 856
Park, Jong-Gu	485, 958	Pereboeva, Larisa	115, 129	Ponder, Katherine P.	34, 162, 857
Park, Julie	148, 819	Pereira, Mario	1003	Ponnazhagan, Selvarangan	434, 619,
Park, Keerang	821	Perera, O. P.	878		746, 886
Park, Kui Lea	453	Perez, Carl F.	476	Ponomarev, Vladimir	258, 983
Park, Man-Seong	1035	Pergolizzi, Robert G.	160, 374, 670, 711	Pope, Melissa A.	139, 1044
Park, Paul J.	1052	Perkinson, Scott	165, 360, 963	Porada, Christopher D.	1052
Park, Sue Nie	84	Perouzel, Eric	487	Porteous, David	479, 480
Park, Yong Serk	472	Perret-Gentil, Marcel	169	Porteus, Matthew H.	709
Parker, Karen	1008, 1009	Perri, Sabrina R.	972	Portlock, Joylette L.	809, 814
Parks, Robin J.	1014	Perricaudet, Michel	285, 967	Porvasnik, Stacey L.	878
Parrott, M. Brandon	1017	Perry, Yaron	671	Potter, David W.	313
Pasanen, Tiina	601, 602, 739	Persons, Derek A.	5, 55, 707	Poulsen, Hans S.	609
Pasceri, Peter	402	Petek, Lisa M.	749	Poulsen, Thomas T.	609
Passineau, Michael	664	Petersen, Denise	177, 408, 696, 905	Praetorius, Mark	516, 541
Passini, Marco A.	210	Peterson, Denise	545, 574	Prasad K. T.	318
Pastan, Ira	130	Peterson, Laura	412	Prell, Rodney A.	578
Pastor, Eric	760	Peterson, Laura J.	913	Prenovitz, Robert	691
Pastore, Lucio	386	Peterson, Timothy E.	392	Press, Oliver W.	354
Patarroyo-White, Susannah	753	Peterson, Daniel A.	1060	Preuss, Meredith A.	1023
Patel, Anita T.	405	Petkar, Animesh	585	Price, Amber	498
Patel, Mahesh	370	Petras, Ognjen	748	Price, Kathleen H.	1031
Patel, Manij	81, 492, 503	Petravicz, Jeremy	64	Priest, Catherine	232
Patel, Swapna	393	Pettersson, Elisabeth	663	Prieto, Jesus	61, 270, 576, 580, 648,
Pater, Cheryl	89	Peuchmaur, Michel	504		978, 980
Pati, Soumya	651	Pfeifer, Alexander	660, 997	Pringle, Ian A.	144, 465
Pattarini, Lucia	197	Pham, Linh D.	306	Printz, Marie	539
Paulson, Henry L.	209, 717	Phillips, M. Ian	928, 929	Prinz, Claudia	706
Payne, Catherine	479	Picard, Virginie	329	Prockop, Darwin J.	514
Payne, Thomas R.	930	Picard-Maureau, Marcus	336	Provost, Nathalie	1050
Peace, Belinda E.	381	Pichon, Chantal	682, 835, 836	Prudhon, Bernard	924
Pearlman, Andrew	921	Pickles, R. J.	687	Przybylowski, Mark	79
Pearse, Damien D.	519	Pickles, Raymond J.	492	Pugatsch, Thea	932
Pechan, Peter	523	Piechaczyk, Marc	724	Puntel, Mariana	1019
Pecorini, Giovanni	896	Pierce, Glenn	89, 103, 425, 752,	Pupi, Juliana	329
Pedersen, Lene	734		753, 1002	Puskovic, Veljko	220
Pedersen, Mikkel W.	609	Pierce, Glenn P.	418	Puthenveetil, G.	702
Pedersen, Nina	609	Pierog, Jaroslaw	512, 925	Puttaraju, M.	712
Pedroso de Lima, Maria C.	592, 842	Pinedo, Herbert M.	288, 594	Pützer, Brigitte M.	622, 943
Pei, Zifei	448	Pintel, David J.	15	Qi, Xiaoping	212
Pelled, Gadi	384, 827	Piovani, Bianca	398	Qian, Cheng	448, 580, 978, 980
Pellegrini, Graziella	182	Pipiya, Teona	278, 292, 690	Qian, Hu Sheng	238, 791
Pellegrini, Vittorio	365	Piscione, Federico	386	Qian, Keping	928, 929
Peller, Leah	394	Pitarresi, Tina M.	138	Qiao, Jian	23, 451, 741
Pellinen, Riikka	601, 739	Plank, Christian	473, 480, 510, 511, 573,	Qin, Min	686
Peluso, Richard	754, 756, 760		846, 935	Qing, Keyun	303, 342, 982
Pen H.	450	Plotz, Paul H.	859	Qiu, Jianming	15
Peng, Baowei	551	Plusquellec, Daniel	481	Qiu, Jianping	558

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Qu, Guang	422, 424, 425	Rehman, Khaja K.	986	Rodicker, Florian	943
Quattrini, Angelo	41	Reier, Paul J.	1055	Rodijk, Mark	286
Quax, Paul H. A.	153, 203	Reik, Andreas	324, 628, 901	Rodriguez, Carlos M.	61
Quenneville, Simon P.	152, 239, 537	Rein, Daniel T.	623, 970	Rodriguez, Ronald	137, 275, 449
Quesnel, Bruno	353, 586	Reineke, Theresa M.	362	Rodriguez-Madoz, Juan R.	576
Qureshi, N.	702	Reiser, Jacob	533	Roe, Mikel	216
Rabelink, Martijn J.	134	Reiser, Jakob	792	Roehl, Holger	83
Raben, Nina	859	Reiss, Russel	193	Roelvink, Peter W.	105, 1020, 1025
Rabinowitz, Joe	1050	Reiter, Kaye	791	Rogers, Pamela	934
Rabinowitz, Joseph E.	17, 19, 91	Religa, Zbigniew	201	Rohll, Jonathan	83, 1062
Rabkin, Samuel D.	351, 798	Remsey-Semmelweis, Ernö	658	Rojas, Virginia	290
Rabussay, Dietmar	556	Ren, Changchun	434, 619, 746	Rokovich, Joe	934
Radcliffe, Pippa	36, 168, 235, 636	Ren, Xianying	991	Rom, William N.	278, 292, 690
Raffel, Corey	62	Renoir, Dominique	285	Roman Zak	12
Rafii, Shahin	196	Rentsendorj, Altan	844	Rommelaere, Jean	684
Ragni, Margaret	1002	Rentz, Sarah S.	322	Roncarolo, M. G.	1004
RaiKwar, Sudhanshu P.	966, 1021	Rethwilm, Axel	336	Roncarolo, Maria Grazia	906
Raizada, Mohan K.	939, 1000	Rettig, Michael	259, 606	Roncarolo, Maria-Grazia	631
Rajagopalan, Sanjay	194	Reyes, Morayma	379	Ropper, Alexander	374
Rajavashisth, Tripathi B.	393	Reyes-Sandoval, Arturo	719	Rosales, Gabriela A.	306
Raki, Mari	960	Reynolds, Ann M.	952	Rosenacker, Joseph	510, 511
Ralph, Scott G.	528, 1062	Reynolds, Paul N.	21, 391, 623, 664, 952	Rosol, Michael	696
Ramakrishnan, Sriram	820	Rhee, Gyu Seek	453	Ross, Colin J. D.	40
Ramamurthy, Poorna	6	Rice, Karen	174	Ross, Joel	1014
Ramesh, Nagarajan	290	Rice, Kevin G.	364, 833, 841	Rossi, Claudia	182, 992
Ramesh, Rajagopal	500, 954, 955, 1008	Rich, Dawn	1008	Rossi, John	410
Ramezani, Ali	186	Richard, Emmanuel	701	Rossi, John J.	366, 367, 548, 549
Ramirez, Anthony	227, 536	Richard, Robert E.	301	Roth, Jack A.	500
Ramirez, Pedro J.	788, 971	Richards, Donald	1009	Roth, Justin C.	914
Ramirez-Correa, Genaro	236	Richter, Johan	900	Rothman, S.	42
Rammensee, Hans G.	562	Richter, Raphaela	277	Rothsten, Jeffrey D.	44
Ramsubir, Shobha M.	868	Richter, Wilfrud	882	Rots, Marianne G.	28, 293, 665, 716
Randlev, Britta	89	Rickert, Markus	882	Rotz, Marianne G.	153
Rando, Thomas A.	810	Ridler, Chris	166	Rousseau, Joel	152, 537
Rao, Koppal S.	342	Riederer, Andreas	95	Rovelink, Peter W.	689
Rao, Madhusudhana N.	318, 482	Rinaldi, Monica	149	Rowe, Peter B.	405
Raper, Steven E.	1053	Rinne, Mikael	282	Rowley, Christine R.	80, 209
Rasko, John	1002	Ritchey, Julie	259	Roy, Himadri	389
Rau, Kun-Ming	295	Rittner, Karola	119	Roy, Krishnendu	838
Ravindran, Sundari	781	Riu, Efren	155, 1039	Roy, Soumitra	128, 1016
Rawlings, David J.	158, 904	Riu, Efren P.	96	Royer, Hans-Dieter	440
Rawlins, Bernard	888	Rius, Denise	527	Rozengurt, Nora	693
Ray, Angelita	1064	Rivara, Michael	689	Rubanyi, Gabor M.	238, 791
Raynald, Roy	1005	Rivara, Michael C.	557	Rubin, Hillard	523
Razavi, Mahmood	1002	Rivera, Angel A.	443, 1023	Rubin, Janet E.	376
Razo, Roberto	320	Rivera, Victor M.	215, 1048, 1053	Rucker, Mary B.	377
Razquin, Nerea	270, 648	Rivière, Isabelle	72, 79, 555, 652	Rudikoff, Andrew G.	915
Razzouk, Sleiman N.	466	Rizzi, Marta	564, 1043	Rudolph, Carsten	510, 511
Reay, Daniel P.	382	Robbins, Paul D.	383, 554, 778, 809,	Rueckriem, Benedikt	25, 995
Reay, Philip	265		896, 986	Rueger, Maria A.	25, 277, 995
Rebar, Edward	107, 302, 1049	Robert, Elodie	701	Ruiters, Marcel H. J.	467
Rebar, Edward J.	531, 649, 659, 931	Robert, Mango	856	Russell, David W.	526, 749
Recchia, A.	1004	Roberto, Cattaneo	26, 593	Russell, Stephen J.	24, 281, 306, 327,
Recchia, Alessandra	46, 60	Roberts, Byron	1058		610, 613, 614, 1024, 1026
Reddy, P. Seshidhar	445	Roberts, Jill M.	751	Rustagi, Pradip	1002
Redmond, Jr, D. Eugene	432	Robins, Paul D.	892	Rutschman, Robert	179
Reese, Jane	1006	Rockenstien, Edward	525	Ruysschaert, Jean-Marie	478

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Ryan, Tom	901	Sano, Takeshi	430, 777	Schmid, Ralph	512, 925
Ryazantsev, Sergey	693	Santiago, Francisco	543	Schmidt, Erika	667
Ryser, Martin F.	915	Santoni de Sio, Francesca R.	2	Schmidt, Manfred	345, 706, 708, 1046
Saamoaoh-Moffatt, Stanley	359	Santulli-Marotto, Sandra	588	Schmidt, Manuel	646, 816
Sabatino, Denise	1002	Saperstein, David	1064	Schmidt, Ulrike	590
Sabatino, Denise E.	37, 345	Sara, Tomasi	188	Schneider, Ayn	699, 867
Sachdeva, Geetanjali	71	Sarah, Chung	695	Schneider, Gabriele	277
Sack, Florian	816	Sarah, Hamm-Alvarez	844	Schneider, Michael C.	474
Sadelain, Michel	72, 555, 652	Sarkadi, Balazs	908	Schneider, Richard M.	614
Sadoui, Naimi	1038	Sarkar, Rita	34, 828	Schneider, Tamar	30, 280
Saeki, Y.	796	Sartori, Daniela	60	Schnepp, Bruce C.	346, 744
Saeki, Yoshinaga	624, 802, 804	Sarukawa, Mutsuko	388	Scholes, J.	702
Saenz, Dyana	10	Sasaki, Erika	910	Schorr, Joachim	142
Sagi, Sreedhar	184, 957, 959	Sasaki, Katsunori	771	Schowalter, David B.	108, 438
Saglio, Giuseppe	149	Sasano, Tetsuo	394	Schreiber, Valerie	119
Saito, Yuji	500, 954	Sasser, A. Kate	92	Schroff, Matthias	816
Saito, Yuka	187	Sato, Katsujiro	244	Schuchman, E.	853
Saitoh, Hiroki	488	Sato, Makoto	975	Schuening, Friedrich G.	404
Saijan, Umadevi	685	Sato, Naoyuki	228, 517	Schuettrumpf, Joerg	750
Sakae, Mitsuko	840	Sato, Toshinori	847	Schughart, Klaus	119
Sakai, Tetsuro	930	Satoh, Eigo	406	Schumacher, Ton N.	269
Sakai, Yoshio	456, 575	Satoh, Takefumi	291	Schumer, Gregory P.	368, 373, 490
Sakamoto, Masamitsu	632	Saudemont, Aurore	353, 586	Schwartz, David A.	676
Sakamoto, Miki	378	Sauer, Julie A.	77	Schwartz, Tim	317
Sakata, Yoichi	343, 427	Saulle, Dwight	889, 890	Schwarz, Bianca	573
Sakiyama, Yukio	175	Sauthoff, Harald	278, 292, 690	Schwarzenberger, Paul O.	605
Sakoda, Tsuyoshi	726	Savontaus, Mikko	118	Schwarzwaelder, Kerstin	706
Sakuma, Ryuta	73	Sawatzki, Sanna	241	Score, Paul R.	823, 1042
Sakurai, Fuminori	113	Sawatzki, Shanna	240, 460	Scott, Jeannine	240
Sakurai, Ken	858	Sawatzki, Shanna M.	243	Scott, Shaun P.	596
Sakurai, Minoru	965	Sawicki, Janet A.	603	Scott, Simon D.	962
Salazar, Felix H.	371	Scallan, Ciaran	102, 103	Seaver, Sally S.	897
Salem, Husein K.	395	Scallan, Ciaran D.	753	Seefried, Stefan	742
Salmon, Andre	924	Scaria, Abraham	523	Segerman, Anna	766
Salte, Ragnar	151	Scaria, Puthupparampil V.	834	Seguin, Johanne	475, 1038
Saltzman, Charles L.	765	Schaack, Jerome	543	Seiler, Michael P.	894
Salva, Maja Z.	180	Schaaf, Axel	184, 957, 959	Seitz, William A.	477
Salvatore, Francesco	386	Schaap, Gerard R.	289	Sellers, Stephanie	708
Salvi, Alessandro	236	Schacter, Pinhas	280	Semenkovich, Clay	856
Sampson, Lisa	578	Schaefer, Mary	556	Sen, Sabyasachi	109
Samulski, Jude	1050	Schaer, Gary L.	194	Senadherra, Dinithi	177, 905
Samulski, R. Jude	19, 91	Schaffer, David V.	20, 401, 547, 725	Sena-Esteves, Miguel	23, 995
Samulski, Richard J.	11, 17, 94, 238	Schagen, Frederik H. E.	59, 121, 289	Senay, Hélène	1005
Sanchez, Andre R.	519	Schalchterman, Alexander	750	Senior, S.	796
Sanchez, Ramon	585	Scheetz, Todd E.	4	Senzer, Neil	1008, 1009
Sanchez-Perez, Luis	352	Scheffler, Bjorn	851	Seok, Ji Hyun	453
Sanchez-Perez, Luis A.	256	Schenk, Andrea	590, 742	Seong, Jin-Sil	946
Sandalon, Ziv	99, 880	Scherer, Lisa J.	548	Seppen, Jurgen	727, 872
Sandberg, Lena M.	825	Scherman, Daniel	475, 506, 883, 1038	Serganova, Inna	983
Sander, Astrid	646	Scherr, Michaela	633	Seroze, Armelle	924
Sanders, Christine T.	1060	Scheule, Ron	473, 507	Serra, Delila	862, 1012
Sandrin, Pierre	682, 836	Schiekofer, Stephan	468	Sessa, Bill	936
Sands, M.	42	Schiffelers, Raymond M.	834	Sessa, William C.	1049
Sanftner, Laura M.	215	Schillinger, Ulrike	510, 511, 573, 846	Sferra, Thomas J.	534, 697
Sanmiguel, Julio	128	Schlachterman, Alex	37	Shackelford, Rodney	538
Sano, Akihiko	830	Schlachterman, Alexander	32, 104, 1047	Shah, Ankur	502
Sano, Renata	698	Schlemmer, Stefanie	573	Shah, Chirag	585

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Shah, Prediman K.	393	Sibley, Don A.	621	Soda, Yasushi	672, 910
Shaish, Aviv	969	Siddiqui, M. A. Q.	658	Soeffker, Diane C.	281
Shan, Z.	1029	Sidi, Ami	30	Soffer, Eric	790
Shani, Noam	921	Siedel, Nancy E.	902	Sohn, Joo-Hyuk	444, 945
Shankar, Veijay	319	Siegal, Gene P.	443, 620, 970, 1023	Sohr, Sooah	422, 424
Shankar, Vijay	580	Sievers, Richard	192	Soifer, Harris	779
Shao-an	450	Signori, Emanuela	149	Soifer, Harris S.	548
Sharma, Rakesh K.	651	Sillevius Smitt, Peter A. E.	114, 286	Soldatenkov, Viatcheslav	617
Sharma, Sherven	686	Simoes, Sergio	842	Soloff, Adam	554
Shavit, Menachem	921	Simoes, Sergio M.	592	Somerton, Luci	720
Shaw, Denise R.	619	Simon, Gregory G.	565	Somia, Nikunj V.	7
Shaw, Kit L.	408	Simonenko, Vera	607	Sommer, Jurg	102, 1002
Shayakhmetov, Dmitry	271, 899	Simonetti, Elles	331	Sommer, Jurg M.	425
Shayakhmetov, Dmitry M.	136, 1010	Simpson, Catherine	979	Sondergaard, Claus S.	734
Shayakhmetovv, Dmitry	22	Simpson, Guy	266, 274	Sondhi, Dolan	432, 1060
Shea, Barry	570	Sinagra, Gianfranco	236	Song, Sihong	106, 429, 761
Shemyakin, Igor G.	843	Singer, Oded	994	Song, Young K.	812
Shen, Hongmei	673	Singh, Charanjit	507	Sonnino, Sandro	870
Shen, Jin Song	858	Singh, Dinesh	458	Sonntag, Jennifer	305
Shen, Leping	929	Singh, Rajkumar Sunil	682	Soong, NayWei	542
Shen, Zhenping	158	Singh, Vimal K.	651	Soria, Claudine	285
Shenouda, Steve	522	Sinigaglia, Milena	197	Soria, Jeannette	285
Shewach, Donna S.	1033	Sinn, Patrick L.	80, 502, 688	Sorrentino, Brian P.	630, 634
Sheykholeslami, Kianoush	1061	Sinzger, Christian	562	Sorrento, Daniela	386
Shi, Juan	625	Sirakawa, Toshiro	598	Sova, Pavel	276
Shiau, Al-Li	579, 595, 599, 961	Sitia, Giovanni	1019	Sowers, Anastasia L.	769
Shiba, Hiroaki	965	Skelton, Dianne	177, 696	Spagnou, Sebastien	321
Shibata, Hiroaki	703	Skelton, Dianne C.	574	Sparks, Kerriann	249
Shido, Koji	196	Skiadopoulos, M. H.	687	Sparrman, Marianne	825
Shieh, Gia-Shing	599, 961	Skimming, Jeffrey W.	247	Spees, Jeffery L.	514
Shigemura, Katsumi	604	Skuk, Daniel	152, 1005	Spencer, Brian J.	700
Shimada, Hiroshi	788	Slack, Mark	803	Spencer, David M.	347
Shimada, Hiroyuki	696	Sladek, Jr, John R.	1060	Spencer, H. Trent	253, 399, 912
Shimada, Takashi	86, 311, 865, 866	Slavin, S.	1004	Spencer, Jacqueline F.	447, 1011, 1018
Shimamura, Munehisa	228, 517	Sleeper, Margaret	855	Spiegel, Martin	590
Shimazaki, Kuniko	388, 758, 1061	Slepushkin, Vladimir	1003	Spindler, Jonathan E.	712
Shimizu, Ayako	1028	Slepushkina, Tatiana	1003	Spratt, Kaye	659
Shimizu, Takashi	272	Sloane, Evan M.	530	Spratt, S. Kaye	931, 107
Shimozato, Osamu	571	Smerdou, Cristian	576	Springfeld, Christoph	593
Shin, Kyung-Sook	611	Smicum, Yoel	381	Squiban, Patrick	252, 577
Shinar, Hadassah	384	Smirnow, Irina	590, 742	Srinivasakumar, Narasimhachar	404
Shinoda, Atsuo	287	Smith, Bruce F.	249	Srinivasan, Rahul	806
Shinomiya, Takashi	348, 584	Smith, C. I. Edvard	150	Srivastava, Anand S.	522
Shinozaki, Katsunori	1035	Smith, Carolina I.	489, 493	Srivastava, Arun	303, 342, 764, 982
Shin-ya, Masaharu	584	Smith, Colin	816	Staber, Patrick D.	209
Shirakawa, Toshiro	604	Smith, Howard	999	Stachelek, Stanley J.	200
Shirohzu, Hisao	495	Smith, Jeffrey S.	464	Staecker, Hinrich	516
Shivakumar, Rama	198, 583	Smith, Leslie A.	199	Staecker, Hinrick	541
Shorrock, Claire	274	Smith, Louis C.	139, 1044	Staerk, Sybille	612
Short, Joshua J.	116	Smith, Rebecca L.	507	Stafford, Darrel W.	31
Shou, Weinian	303, 982	Smith, Theodore A. G.	666	Stagg, John	349, 911
Shou, Yan	630	Snell, Jeffrey	194	Stähli, Stefan	663
Shuahua, Huang	213	Snoeys, Jan	120, 387, 563	Stamatoyannopolous, George	899
Shuanglin, Hao	213	Snowden, Andrew W.	325, 326, 649	Stamatoyannopoulos, George	1001
Shukla, Shruti	976	Snyder, Chris W.	875	Stanley, Hilary A.	43, 211
Shumilla, Jennifer A.	530	Snyder, Richard O.	420	Stargel, Amanda	1013
Siapati, Elena K.	166	Sobol, Robert E.	1007	Stayton, Patrick S.	639

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Steck, Eric	882	Sullivan, Mark A.	552	Tamiya, Takashi	47
Steer, C. J.	178, 985	Sullivan, Sean M.	851, 941	Tan, Mengqun	303
Steer, Clifford J.	154, 216	Sumazaki, Ryo	272	Tan, Siyuan	233, 302, 324, 325
Stefani, Anna-Lisa	597	Sumimoto, Hidetoshi	1028	Tan, Yadi	372, 567
Stefanski, Jolanta	652	Summer-Jones, Stephanie G.	143, 494	Tanaka, Motoyoshi	845
Stehle, Thilo	111	Summers, Colin	939, 1000	Tanaka, Naoko Iida	840, 847
Stein, Colleen S.	171, 1059	Sun, Baodong	339, 699, 867, 1054	Tanaka, Nobushige	1057
Steinau, Hans Ulrich	787	Sun, Huijie	383	Tanaka, Ryuichi	254
Steinert, Andre F.	891	Sun, Jian-Sheng	316	Tanaka, Toshihiro	771
Steinstraesser, Lars	787	Sun, Junwei	345	Tanase, Diana	439, 532, 735
Stemberger, Axel	846	Sun, Kevin	625	Tang, Hao	1048
Stepanova, Lilia	630, 634	Sun, Liang	212	Tang, Mei	429
Stephan, Matthias	72	Sun, Minli	390, 848	Tang, Qiushi	169
Stephan, Sharon A.	77	Sun, Yuansheng	614, 740	Tang, Susan	542
Stephen, Russell J.	684	Suominen, Elina	118	Tang, Waixing	218
Stern, Baruch	921	Surace, Enrico M.	647	Tang, Yao Liang	928, 929
Stern, David M.	468	Suroskey, Richard	752	Tang, Yi	928
Stevenson, Susan C.	666	Suroskey, Richard T.	89	Tani, Kenzaburo	582, 672, 910
Stewart, Duncan J.	198	Sutjipto, Suganto	781	Taniguchi, Naonori	604
Stewart, Gregory R.	210	Suzuki, Eiji	572	Tanioka, Yoshikuni	910
Stewart, Sandra	476	Suzuki, Satoru	311	Taniyama, Yosiaki	191
Stiger, L. B.	146	Svahn, Mathias G.	150	Tanner, Alice K.	202
Stitz, Joern	333	Swanson, Debra	279	Tanswell, Keith	685
Stoddard, Barry L.	1030	Sweeney, Colin	1006	Tanzi, Giancarlo	732
Stodola, Tom	476	Swisher, Stephen G.	675	Tao, Kesheng	861
Stokes, Rebecca	405	Sylvain, Michel	1005	Tao, Lucy	775
Stoltz, Donna B.	812	Sylvester, Karl G.	308	Tarantal, Alice F.	377, 681, 984, 988
Stone, Daniel	123, 271	Szalai, Alex J.	1013	Tarbiyat-Boldaji, Mehrdokht	635
Storgard, Chris M.	1024	Szeto, Gregory	763	Tardy, Claudine	868
Storm, Gert	834	Szymanski, Paul	238	Tasciotti, Ennio	600
Storm, Theresa	13, 337	Tabata, Toshiaki	495, 703	Tatalick, Laurie M.	489, 881
Storm, Theresa A.	96, 172, 371	Tabucchi, A.	1004	Tatsis, Nia	719
Stornaiuolo, Anna	46, 398	Taft, Peter	765	Tatsuno, Gwen	752
Stout, Timothy	1064	Tafuro, Sabrina	197	Tatsuzawa, Osamu	175
Straface, Giuseppe	896	Tagawa, Masatoshi	571	Tavakoli, Reza	925
Strassheim, Lisa M.	892	Tahara, Minoru	711	Taylor, Doris A.	927
Strauss, Bryan E.	85, 417	Tahimic, Candice G. T.	642	Tebbets, Jessica	246
Strauss, Robert	276	Tai, Kuo-Feng	1045	Tedford, Nathan C.	850
Strawn, Tammy	247	Taichman, Lorne B.	657	Teh, Bin S.	291
Strayer, David S.	370, 544, 559, 917	Taira, Kazunari	771, 1028	Tejada, Rafael	196
Streetz, Konrad L.	308, 371	Takada, Satoshi	845	Telenius, Adele	476
Strom, Ted S.	179	Takahashi, Masafumi	758	Tellez, Joe	1052
Strong, Theresa V.	619	Takahashi, Masayo	996	ten Haaff, Mark R.	423
Su, Dan	1008	Takahashi, Satoru	330	Tenase, Diana	45
Su, Hua	192	Takahashi, Satoshi	672	Tenenbaum, Liliane	641
Su, Su	86	Takahashi, Tsuneo A.	672	Teng, Qingshan	439, 532
Suda, Tetsuji	991	Takakuwa, Yukiko	866	Teng, Qingshang	45
Sugimoto, Khoji	604	Takatsy, Zsuzsanna	807	Teng, Qinshan	735
Sugimura, Kazuro	604	Takayama, Koichi	620, 629, 960, 964	Ten hunen, Anni	739
Sugiura, Wataru	406	Takeda, Shin'ichi	244, 378	Teo, Wu-Lin	335
Sugiyama, Osamu	887	Takeshita, Fumitaka	830	ter Horst, Maarten	286
Suh, Beom-Seok	84, 946	Takeuchi, Koichi	388, 1061	Teramoto, Kenichi	406
Suh, David A.	105	Takino, Naomi	49, 1051	Terao, Keiji	49, 703
Sukonnik, Tanya	402	Taksir, Tatyana V.	210	Terao, Shiji	598
Sullenger, Bruce	202, 903	Tal, Jacob	801	Terao, Shuji	604
Sullenger, Bruce A.	611	Tam, Orlena	445	Tertilt, Christine	557
Sullivan, Mark	222	Tamin, Azai	554	Terzic, Andre	392

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Teshale, Habte F.	638	Topp, Max S.	355, 562	Ulrich, Kristina	487
Tessitore, Alessandra	698	Torne-Celer, Caroline	743	Umezawa, Akihiro	858
Themis, Micahel	235	Tornquist, Elisabeth	150	Unev, James	636
Themis, Michael	166	Toth, Karoly	1011, 1018	Ungerechts, Guy	9
Themis, Mike	36	Totoiu, Minodora	232	Unwalla, Hoshang J.	366
Theopold, Chris	987	Tourkova, Vilna	258, 983	Urabe, Masashi	419
Theopold, Christoph F. P.	797	Toussaint, Nathalie Y.	851	Urano, Fumi	1051
Theseling, Carmen C.	943	Townes, Tim	901	Urasawa, Nobuyuki	244
Thies, Scott	232	Townsend, Kay	82	Urbinati, Fabrizia	403
Thomaidis, Annick	285	Tran, Diane Thi	4, 171	Urnov, Fyodor	649, 714
Thomas, Clare	459	Trapnell, Bruce C.	457	Urnov, Fyodor D.	713
Thomas, Suzanne	265	Traver, Geri L.	768	Usas, Arvydas	385, 632, 885, 895
Thomasson, Bobbie	1, 704, 913	Tremblay, Jacques P.	152, 239, 537, 1005	Usui, Joichi	330
Thompson, Arthur R.	158	Tremble, Patrice	934	Uzuka, Takeshi	189, 190, 926, 933, 938
Thompson, Bonne	39	Trifari, Sara	906	Vacante, Dominick	536, 539
Thompson, Brad	1027	Trimarco, Bruno	386	Valentine, Vincent G.	514
Thompson, Christine S.	999	Triozzi, Pierre L.	124, 619, 664	Vallanti, Giuliana	375
Thompson, E. Brad	477	Trochon-Joseph, Veronique	285	van Bekkum, Dirk	770
Thompson, Jesse	913	Trojan, Lutz	957, 959	van Beusechem, Victor W.	59, 121, 130,
Thompson, Jill	23, 256, 352	Trono, Didier	170, 409, 900	288, 289, 463, 594	
Thompson, Timothy C.	291, 356	Trucco, Massimo	986	van den Boom, Marly	269
Thorrez, Lieven	413	Trzpis, Monika	467	van den Doel, Petra B.	463
Thrasher, Adrian	36, 166	Tsang, Tom	195	van den Oever, Karen	40
Throm, Stacy L.	55	Tsang, Tom C.	263	Van den Wollenberg, Diana J.	134, 261
Thule, Peter M.	376, 893	Tsipouras, Petros	381	van der Gun, Ieneke T. F.	467
Thyagarajan, Bhaskar	814	Tsuchida, Junko	49	van der Heijdt, Stephanie	132, 134
Tian, Baohong	322	Tsuchiya, Naoto	254	Van der Voort, Ellen I.	261
Tian, Jie	464	Tsuchiya, Shigeru	906	van Deutekom, Judith C. T.	638
Tian, Kian W.	750	Tsugumine, Shu	720	van Eert, Simone J.	293
Timiryasova, Tatyana	807	Tsujino, Takeshi	726	van Geer, Michael A.	130
Timme, Terry L.	291, 356	Tsukada, Hideo	49	van Heeckeren, Anna	829
Timon, Marcos	816	Tsumoto, Kohei	63	van Heeckeren, Christiaan	829
Timonen, Pekka	739	Tsuruta, Yuko	129, 623	Van Linthout, Sophie	387, 563
Tina, Rogers	322	Tu, Guang	1058	van Ommen, Gert-Jan B.	638
Tjelle, Torunn Elisabeth	151, 157	Tubb, Julie	1001	Van Oosten, Rebecca	944
Todo, Tomoki	804	Tuddenham, Edward G. D.	36	Van Roey, Melinda	290
Toes, Rene E.	261	Tufaro, Frank	627	Van Rooijen, Nico	413, 862
Tohse, Noritsugu	933	Tulimaki, Kirsi	739	van Waarde, Aren	716
Toita, Nariaki	175	Turgeman, Gadi	384	van Wart Hood, Jill	1008
Tojo, Arinobu	672	Turnbull, Emma	265	van Weel, Vincent	153
Tokusumi, Yumiko	723	Tuszynski, Mark	527	Vandenbergh, Luk H.	88
Tollefson, Ann E.	447, 1011, 1018	Tvrdik, Wayne A.	142	VandenDriessche, Thierry	413
Toloza, Eric M.	611	Twisk, Jaap	40, 423	Vanderbyl, Sandra	476
Tom, Susan	514	Tyminski, Edyta	802	Vanhook, Miriam K.	503
Tom, Susan S.	540	Tzai, Tzong-Shin	599	VanRoey, Melinda	445, 578, 1058
Tomasi, Sara	197	Ubhayakar, Kiran	159, 643	VanRoey, Melinda J.	974
Tomita, Hideyuki	287	Uchida, Hiroaki	771, 926, 933	Varathalingam, Anusha	143, 465
Tomita, Naruya	517, 940, 1036	Uchimaru, Kaoru	672	Vargaftig, B. Boris	506
Tomita, Tetsuya	1036	Uchino, Junji	629, 964	Vargas, Joe	102
Tomizuka, Kazuma	996	Ueda, Kyouji	703	Varghese, Susan	351
Tomonari, Akira	672	Ueda, Yasuji	495, 703	Varner, Kurt J.	738
Tone, Catherine M.	273	Uemura, Hirotugu	845	Vasi, Jozsef	825
Tong, Alex	1009	Ueno, Hikaru	938	Vassallo, Elizabeth	432, 1060
Tong, Alex W.	1008	Ugen, Kenneth	948	Vassilopoulos, George	526
Tong, Suxiang	730	Uil, Taco G.	132	Vasu, Chenthamarakshan	116
Tonnelle, Cecile	54	Ujhelly, Olga	908	Vatamaniuk, Marko	1053
Tonon, Giancarlo	149	Ulmer, Jeffrey B.	556	Vekemans, Katrien	387

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Veldwijk, Marlon R.	344	Wait, Richard B.	658	Watkins, Linda R.	530
Vellekamp, Gary	781	Wakeling, Madeleine	265	Watson, Deborah J.	520
Vellinga, Jort	132, 134, 663	Walker, David G.	596	Wattiaux, Robert	140
Velu, Thierry	641	Walker, Henry J.	77	Wattiaux-De Coninck, Simone	140
Venneri, Mary Anna	332	Walker, John	259	Waud, William W.	618
Venstrom, Kristine	107	Walker, Jon E.	606	Weber, James	808
Vera, Maria	61, 270, 648	Walker, Taylor	234	Weber, James J.	156
Verbart, Dennis	40, 423	Walker, Wendy E.	484	Weber, Wilfried	715
Verheesen, Peter	423	Wallach, David	969	Webster, Jack M.	48
Verhoeven, Michel	204	Walmsley, Lucy	735	Webster, Paul	363
Verissimo, Paula	842	Walmsley, Lucy E.	208, 235, 532, 1062	Wechuck, James B.	794
Verkman, Alan S.	678	Walter, Glenn A.	251	Wegman, Troy R.	77
Verma, Inder M.	525, 700, 994, 1057	Walters, Stacey N.	405	Wei, Lisa	993
Verma, Yogesh K.	651	Walton, Raquel M.	520	Wei, Lisa L.	57, 1064
Veronika, von Messling	26, 593	Wang, Alfred Y.	172	Wei, Ming	499
Verstegen, Monique M. A.	1046	Wang, Chiou-Miin	697	Wei, Ming Q.	596
Vertin, Beth	999	Wang, Chuansong	534, 697	Wei, Wen-Chi	550
Vich, Jeronia	44	Wang, Daowen	428	Wei, Xin	428
Vider, Jelena	258, 983	Wang, Guoshun	415, 514	Wei, Zhangyong	218
Vigna, Elisa	332	Wang, Haili	882	Weigel-Kelley, Kirsten A.	764, 982
Vigneron, Jean-Pierre	504	Wang, Hongjie	899	Weinberg, Marc	549, 553
Vijay, Sharmila	102	Wang, Hongyu	356	Weiner, Ashley A.	27
Vila-Coro, Antonio J.	816	Wang, Jeng-Hwan	550	Weiner, Daniel J.	341, 490, 692
Vilalta, Adrian	486	Wang, Jianhua	1047	Weiner, David B.	948
Vile, Richard G.	23, 256, 352, 451, 741	Wang, Jianxiang	291	Weinreich, Michael	299
Villegas-C., Hilda	320	Wang, Jinhui	448	Weinreich, Michael A.	53
Vinogradov, Serguei V.	852	Wang, Jun	712	Weiss, Daniel J.	514, 691
Vishwanath, Reena	548	Wang, Li	469	Weiss, Jonathan M.	583
Visigalli, Ilaria	41	Wang, Lili	161, 569, 692	Weissig, Volkmar	683
Visser, Birgit M.	261	Wang, Lin	978	Welham, Deborah A.	100
Visser, Trudi P.	1046	Wang, Lixin	33, 185	Wellman, Jennifer	89, 752
Vite, Charles	223	Wang, Minghui	443, 620, 623, 664, 970,	Wellner, R. B.	1029
Vlachaki, Maria T.	291		1023	Welsh, Michael J.	110
Vockley, Jerry	438	Wang, Pei-Rong	526	Wen, Jianping	167
Vogler, C.	42	Wang, Peiyin	791	Wenning, Laura	362
Vohra, Seema	585	Wang, Ping	855, 857	Wenstrup, Richard J.	381
Vollmar, Stefan	25	Wang, S.	1029	Wenz, Frederik	344
Vollmer, Regis	812	Wang, Sam	64	Wepsic, H. Terry	635
Vollrath, Douglas	1056	Wang, Su-Jane	550	Wepsic, Terry	585
Volpe, Gisella	149	Wang, Suming	538	Wesseling, John G.	130, 784
Volpi, Nicola	182	Wang, Wenzheng	351	West, Lori	861
von Kalle, Christof	345, 706, 708, 1046	Wang, Xiaofei	422, 424	West, M.	499
von Laer, Dorothee	977	Wang, Xuli	828	Westfall, Denise	83
von Messling, Veronika	328	Wang, Yaming	380	Wexel, Gabi	846
Von Seggern, Dan J.	127, 461	Wang, Yaohe	284, 296, 608, 979	Whalen, Robert G.	542
Vongpunsawad, Sompong	24	Wang, Zhaoxia	1012	Wheeler, Matthew	1043
Vrahlas, Mark S.	891	Wang, Zhong	763, 986	Wheeler, Thurman M.	810
Vu, John	142	Wang, Zhoung	655	White, Lindsay R.	745, 767, 1014
Vukelja, Sasha	1009	Wangensteen, Kirk J.	154	Whiting-Theobald, Narda	915
Waddington, Simon N.	36, 166, 235	Wardwell, Scott	1048	Whitley, Chester B.	871
Wadell, Göran	766	Wasif, Nabil	338	Wickham, Thomas J.	689, 1020, 1025
Wade-Martins, R.	796	Wasserfall, Clive	250, 429	Widera, Georg	556
Wadsworth, Samuel	523	Watanabe, Atsushi	311, 866	Wiedmann, Kathrin	573
Wagemaker, Gerard	1046	Watanabe, Atsushi	86	Wiehle, Sandra	359
Wagner, Denisa	160	Watanabe, Sumiko	910	Wieloch, Peter	882
Wahlfors, Jarmo	601, 602, 739	Watanabe, Takashi	611	Wienhard, Klaus	995
Wahlin, Anders	766	Watchko, Jon F.	382	Wieseler-Frank, Julie L.	530

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Wikman, Maria	663	Wright, Fraser J.	424	Yam, Priscilla	369
Wilber, Andrew	474	Wright, J. Fraser	425	Yamagata, Shizuko	1028
Wilber, Andrew C.	824, 1042	Wright, Nick	166	Yamaguchi, Teruhide	113
Wilcox, David A.	81, 919, 920	Wu, Chao-Liang	579, 595, 599, 961	Yamamoto, Kei	940
Wildhaber, Barbara	170	Wu, Chien-Ming	637	Yamamoto, Keiji	388
Wilkes, Fraser	528, 1062	Wu, Di	128	Yamamoto, Masato	293, 442, 620, 782, 786, 788, 960, 971
Wilkes, Fraser J.	168	Wu, Eric	382		
Willer, Mette	591	Wu, George Y.	381	Yamamoto, Naoki	406
Williams, B. J.	621	Wu, Jerry	369	Yamamoto, Shinji	802
Williams, Christopher C.	635	Wu, Jian	677	Yamanaka, Ryuya	254
Williams, Jim	74, 818	Wu, Jian Liang	650	Yamasaki, Keita	940, 1036
Williams, John	496	Wu, Lily	975	Yamashita, Kouhei	176
Wilson, Alistair	227	Wu, Lizhe	561	Yamazaki, Yohji	965
Wilson, David C.	457	Wu, Lizhi	780	Yan, Hua	406
Wilson, Deborah R.	500	Wu, Qi	640, 644, 1047	Yan, Ziying	754, 756, 760
Wilson, George D.	962	Wu, Qinghui	264	Yanaga, Katuhiko	965
Wilson, James	732	Wu, T. C.	268	Yanagie, Hironobu	840
Wilson, James M.	88, 90, 128, 161, 218, 338, 341, 368, 373, 490, 498, 558, 568, 569, 692, 1015, 1016, 1048, 1053	Wu, Weiwei	974	Yang, Bin	102
Wilson, Linda D.	881	Wu, Xiaolin	13, 817	Yang, Catherine	416
Winkel, Andreas	384	Wu, Xiuju	780	Yang, Guang	291, 356
Winkeler, Alexandra	25, 277, 803, 995	Wu, Zheng	500	Yang, Jiong	1022
Wirthlin, Louisa	898	Wuertzer, Charles A.	552	Yang, K.	521
Wisse, Eddie	387	Wuisman, Paul I. J. M.	289	Yang, Linda	759
Wissler, Manuela	706	Wu-Prior, Wei Wei	1058	Yang, Ning-Sun	550
Witlox, M. Adhiambo	289, 594	Wybranietz, Wolfgang	742	Yang, Pingar	640, 644
Wittig, Burghardt	646, 816	Wybranietz, Wolfgang A.	590	Yang, PingAr	1047
Wixon, Jo	981	Xenariou, Stefania	473, 480, 935	Yang, Xiaoyu	781
Wiznerowicz, Maciej	900	Xia, Dengfeng	931	Yang, Yanping	712
Wognum, Albertus W.	1046	Xia, Deng-Feng	107	Yang, Zihong	847
Wojtkiewicz, Gregory	832	Xia, Haibin	207, 717	Yang, Zuocheng	303, 982
Wold, William S. M.	447, 1011, 1018	Xia, P.	702	Yano, Juliana	393
Wolf, Eckhard	660, 997	Xia, Qinghua	449	Yant, Stephen	826
Wolfe, Darren	213, 214, 220, 806	Xiao, Hong	309	Yant, Stephen R.	148, 815, 817, 819
Wolfe, Darren P.	219, 794	Xiao, Shuifang	1061	Yao, Feng	797, 987
Wolfe, John	223	Xiao, Weidong	625, 747	Yao, Shuyuan	402
Wolfe, John H.	217, 340, 520, 1063	Xiao, Xiao	14, 38, 655, 763, 986	Yaohe	450
Wolfgang, William J.	48	Xie, Jiansong	844	Yasutomi, Kakei S.	584
Wolkers, Monika C.	269	Xie, Litao	4, 171	Yazaki, Takahito	287
Won, Young Hyuk	453	Xiong, Weidong	459, 790	Ye, Chaoyang	15
Wong, F. E.	546	Xu, Fang	862, 869	Ye, Hongqing Q.	67, 728
Wong, K. K.	393	Xu, Hui	1040	Ye, Peiqing	158
Wong, Liang-Fong	208	Xu, Lei	659	Ye, Xiaojing	38
Wong, M.	42	Xu, Li	542	Ye, Xin	242
Wong, Norman C. W.	745	Xu, Lingfei	34, 162, 856	Yechoor, Vijay	39
Wong, Philip	216	Xu, Lingfie	855	Yee, J.-K.	702
Wong-Staal, F.	546	Xu, Ray A.	625	Yee, Jiing-Kuan	367, 369, 414
Woo, Savio L. C.	1035	Xu, Sue	625	Yehling, Eric	222
Woo, Seonock	645	Xu, Xin	640	Yellayi, Srikanth	223
Wood, Patrick M.	519	Xu, Yan	70	Yen, Roy D.	677
Woodle, Martin	834	Xu, Yuhong	551, 839	Yendluri, Satya	974
Worgall, Stefan	557, 689	Xu, Zhuping	15	Yendluri, Satyasri	1058
Working, Peter	290, 445	Xue, Guanhua	390	Yin, W.	178
Wozniak, D.	42	Xue, Shao-an	608	Ying, Baoling	447
Wright, Fraser	422	Yafeng, Zhu	482	Yip, Ping K.	208
		Yajima, Naoki	969	Yi-Sun, Mei	265
			496	Yla-Herttuala, Seppo	389
			254	Yo, Chiah Yian	297

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

### Abstract Author Index

Yo, Yi-Te	579, 599	Zaratiegui, Mikel	61, 648	Zhao, Zuowei	45
Yoder, Mervin C.	52, 303, 342, 982	Zaupa, Cecile	805	Zheng, C.	1029
Yokota, Toshifumi	378	Zeller, W. Jens	344, 916	Zheng, Yi	693
Yokoyama, Takashi	876	Zeng, L.	702	Zhi, Yan	373, 558, 568, 569, 1016
Yoo, Ji Young	84	Zeng, Yue	135	Zhong, Li	52, 303, 342, 982
Yoo, Jinsang	951	Zentilin, Lorena	16, 197, 236	Zhong, Xiaohong	325
Yoon, A-Rum	945	Zern, Mark A.	677	Zhong, Xiaohong	931
Yoshida, Hisahiro	330	Zhang, Baigen	390, 848	Zhou, Chun	48
Yoshida, Jukei	175	Zhang, C.	1029	Zhou, Fen	1023
Yoshida, Kimiko	949	Zhang, Guisheng	812	Zhou, Ling	158, 904
Yoshida, Teruhiko	949	Zhang, H. Steven	931, 325	Zhou, Qin	834
Yoshikawa, Hideki	1036	Zhang, Hao	390, 848	Zhou, Shangzhen	89, 102, 418, 753
Yoshikawa, Hiroyuki	343	Zhang, Haojiang	990	Zhou, Shang-zhen	752
Yoshimitsu, Makoto	861	Zhang, Haoyue	339, 699, 867	Zhou, Xiaoyang	90
Yoshimura, Madoka	244, 378	Zhang, Huame	1012	Zhou, Yangsheng	48
Yoshimura, Naoki	884	Zhang, Huang-Ge	1047	Zhu, Hua	104
Yoshioka, Toru	388, 421	Zhang, Jian	763	Zhu, Qili	904
Yoshizaki, Mariko	723	Zhang, Jian G.	585	Zhu, Tong	38, 655
Yotnda, P.	521	Zhang, JianGang	635	Zhu, Wei	888
Yotnda, Patricia	785	Zhang, Jingli	67	Zhu, Xiaodong	14
You, Changxuan	257	Zhang, Jiwei	390	Zhu, Xiaohong	291
You, Hong	587, 762, 799, 800	Zhang, Jun	956	Zhu, Zen-Bian	664
You, Jun	721	Zhang, L.	687	Zhu, Zeng B.	443, 1023
Young, James W.	555	Zhang, Lei	107, 302, 325, 531, 556, 649,	Ziady, Assem G.	358, 829, 832
Young, Jennifer L.	203	Zhang, Li	659, 931	Ziegler, R. J.	694, 853
Young, Stuart W.	194	Zhang, Liang N.	617	Zilberman, Yoram	384, 827
Yu, De-Chao	290, 445, 775	Zhang, Ling	760	Zimmerman, Heather	67
Yu, Jiang	785	Zhang, Liqun	686	Zinn, Kurt R.	1013
Yu, Julie H.	725	Zhang, M.	975	Zirger, Jeff	459
Yu, Jun	936, 1049	Zhang, Ming	499	Zirger, Jeffrey M.	1019
Yu, Ling	571	Zhang, Nan	1022	Ziying Yan	12
Yu, Seung Shin	407	Zhang, Neal	990	Zoghbi, Huda Y.	717
Yu, Seungshin	396	Zhang, Ping	754	Zolotukhin, Irene	250, 251
Yu, Shaohua	1047	Zhang, Qiuwang	934	Zolotukhin, Sergei	420
Yu, Xiao-Jin	177	Zhang, Steve	198	Zoltic, Phillip W.	56
Yu, Ying	369, 414	Zhang, Steve H.	107, 302	Zoltick, Phillip W.	200
Yuan, Jianda	555	Zhang, Tai-Ping	649	Zori, Roberto T.	878
Yuan, X. Q.	521	Zhang, Tong	31	Zou, Jianxiang	750
Yuasa, Katsutoshi	244, 378	Zhang, Wenge	263	Zou, Weiguo	448
Yue, Yongping	15, 247	Zhang, Xian-Yang	542	Zumstein, Lou	1011
Yulong Zhang	12	Zhang, Xian-Yang	499	Zumstein, Louis A.	1007
Yun, Chae-Ok	84, 441, 444, 581, 945, 946	Zhang, Xiao-Bing	533	Zwick, Michael B.	542
Yun, Ji-Eun	821	Zhang, Y. Clare	704		
Zabala, Maider	580, 978	Zhang, Yi	928, 929		
Zabner, Joseph	110, 566, 760, 765, 768	Zhang, Youming	341		
Zaboikin, Michail M.	404	Zhang, Yuan	468		
Zaboikina, Tatiana N.	404	Zhang, Yulong	1008, 1009		
Zacchigna, Serena	188, 197, 236	Zhang, Zilai	756		
Zaia, John	367	Zhao, Chunxia	448		
Zaia, John A.	369	Zhao, Huizhi	428		
Zaiss, Anne K.	745, 1014	Zhao, Qi	693		
Zajac, Allan	640, 644	Zhao, Wei	210		
Zak, Roman	756	Zhao, Weihong	747		
Zaldumbide, Arnaud	261	Zhao, Yanbing	303		
Zanetti, Maurizio	564, 1043	Zhao, Yidan	588		
Zanjani, Esmail D.	1052	Zhao, Yonggang	198		
Zaoui, Karim	345		551		

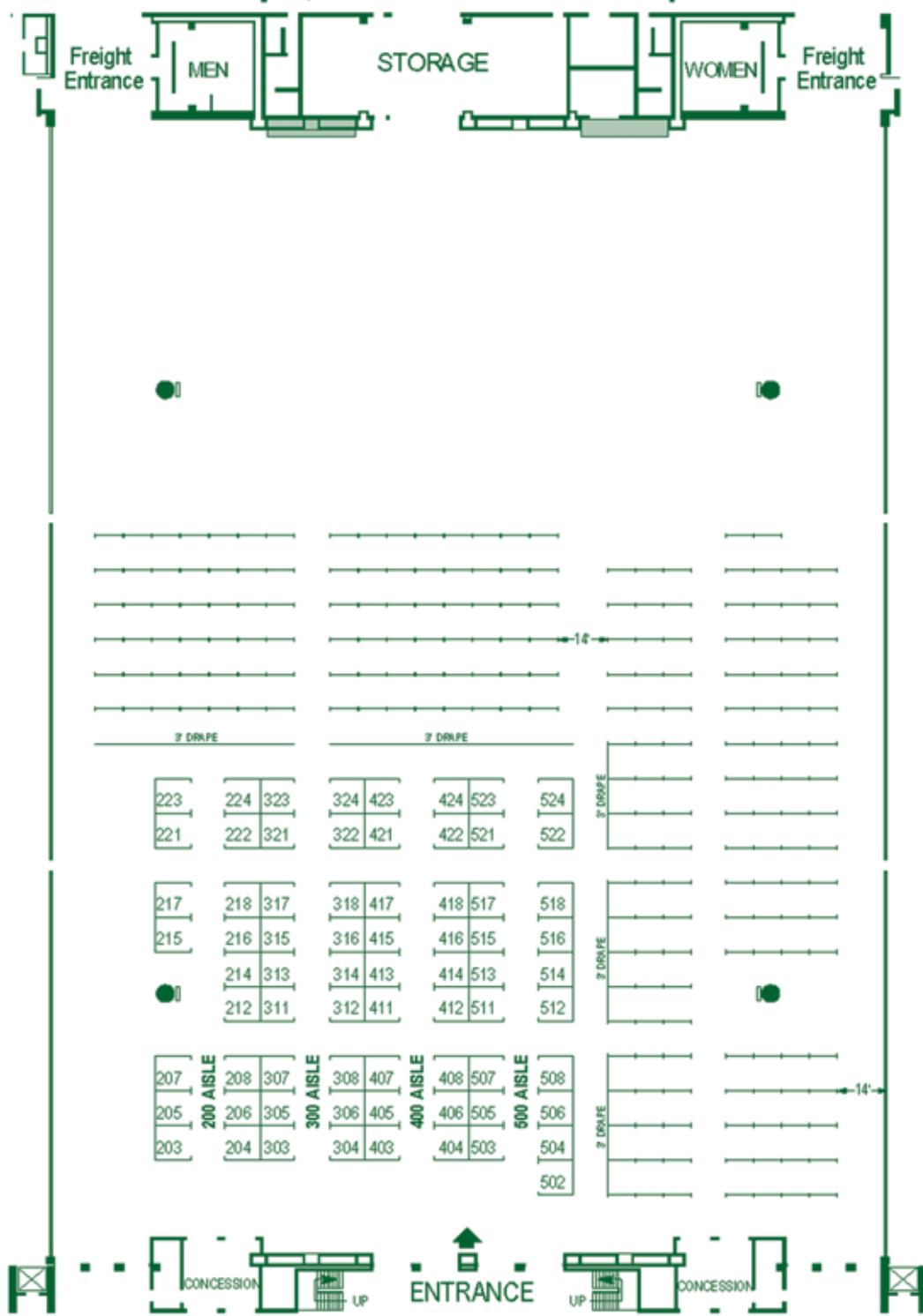
# The American Society of Gene Therapy's 7th Annual Meeting final program

2004

## ASGT 7th Annual Meeting

June 2-6, 2004

Minneapolis Convention Center - Exhibit Hall D



# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

## Exhibitor Information

### General Information

The Exhibit Hall is located in Hall D of the Minneapolis Convention Center (Level One). Please allow adequate time in your daily schedule to visit the exhibits. The companies and organizations exhibiting at the ASGT Annual Meeting will provide you with the latest information on the products and services directly related to your professional needs.

### Exhibit Hall Hours

Thursday, June 3	3:00 pm – 7:30 pm <i>(Welcome Reception begins at 4:00 pm)</i>
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Saturday, June 5	3:00 pm – 7:00 pm <i>(Networking Reception begins at 4:00 pm)</i>

### Exhibitor Registration Desk

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Fax: 49-227-99799-499  
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### American Society of Gene Therapy

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Fax: 414-276-3349  
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[www.asgt.org](http://www.asgt.org)

#### Booth Number: 315 & 317

Stop by the ASGT membership booth to pick-up membership applications and meeting related items. You can also purchase an attractive ASGT shirt and find out how to access the Members' Only Section of the website, including the ASGT on-line membership directory.

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### AppTec Laboratory Services

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E-Mail: [info@arkios.com](mailto:info@arkios.com)  
[www.arkios.com](http://www.arkios.com)

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# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

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Fax: 205-663-0756  
E-Mail: [trevor@avantilipids.com](mailto:trevor@avantilipids.com)  
[www.avantilipids.com](http://www.avantilipids.com)

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Fax: 508-616-0476  
E-Mail: [sshaffer@bioprocessintl.com](mailto:sshaffer@bioprocessintl.com)  
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#### **Booth Number: 306**

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#### **BioSpherix, Ltd.**

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Phone: 315-387-3414  
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E-Mail: [sales@biospherix.com](mailto:sales@biospherix.com)  
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#### **CellGenix, GmbH**

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Fax: 847-395-0808  
E-Mail: [cellgenixusa@aol.com](mailto:cellgenixusa@aol.com)  
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Fax: 978-635-2476  
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[www.corning.com/lifesciences](http://www.corning.com/lifesciences)

#### **Booth Numbers: 422 & 424**

Corning Incorporated, a diversified technology company, manufactures high quality, high performance research products for life sciences. Corning will exhibit reliable and fully-tested cell culture products for scale up. Included will be CellSTACK™ Culture Chambers and E-Cube™ Culture System. Key products from first culture to high throughput screening will also be on display.

#### **Elim Biopharmaceuticals, Inc.**

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Phone: 510-783-1112  
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[www.genassessinc.com](http://www.genassessinc.com)

#### Booth Number: 505

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Germany  
Phone: 49-941-942-760  
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#### Booth Number: 512

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[www.lark.com](http://www.lark.com)

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# 2004

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#### Booth Number: 515

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### MDS Pharma Services

2350 Cohen Street  
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H4R 2N6  
Canada  
Phone: 514-333-0033  
Fax: 514-333-8861  
E-Mail: [info@mdsps.com](mailto:info@mdsps.com)  
[www.mdsps.com](http://www.mdsps.com)

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Waltham, MA 02451  
Phone: 888-735-8437  
Fax: 617-923-8080  
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[www.mjr.com](http://www.mjr.com)

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## final program

# 2004

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E-Mail: lrubin@iupui.edu  
[www.ngvl.org](http://www.ngvl.org)

#### Booth Number: 523

The NGVL consist of three vector production facilities located at Indiana University, Baylor College of Medicine, and the City of Hope/Beckman Research Institute. Two pharmacology/toxicology centers have been established at the University of Florida and Southern Research Institute. The production sites will generate clinical grade adenoviral, adeno-associated virus, DNA plasmid, lentivirus, herpes simplex virus, and retrovirus vectors for Phase I/II gene therapy protocols, while the Pharmacology/Toxicology centers will provide support for investigators needing such studies for their clinical protocols. Requests for vector production and/or pharmacology/toxicology support will be reviewed by the Scientific Review Board and Steering Committee of the NGVL with selection based upon scientific merit, feasibility and availability of NGVL resources. If the application is approved through the Steering Committee, clinical grade materials and pharmacology/toxicology studies will be provided without charge for human gene therapy trials. An application packet can be downloaded from the NGVL website at <http://www.ngvl.org> or obtained through the Coordinating Center.

#### National Institutes of Health

##### National Heart, Lung, and Blood Institute

6701 Rockledge Drive, Room 10183  
Bethesda, MD 20892-7956  
Phone: 301-435-0499  
Fax: 301-480-2849

#### Booth Number: 316

The Programs of Excellence in Gene Therapy (PEGTs) have been established by the National Heart, Lung, and Blood Institute to facilitate clinical gene therapy studies by the multidisciplinary collaboration of investigators skilled in the application of gene therapy for any one or combination of cardiovascular, pulmonary, and hematologic diseases. The PEGTs also provide specialized services, such as generating vectors for clinical use, performing morphologically based studies, procuring and processing hematopoietic stem cells, and offering to perform primate transplantation studies.

#### Nature Publishing Group

345 Park Avenue  
New York, NY 10010  
Phone: 212-726-9200  
Fax: 212-699-9591  
E-Mail: [a.seda@natureny.com](mailto:a.seda@natureny.com)  
[www.nature.com](http://www.nature.com)

#### Booth Number: 412

Nature Publishing Group publishes distinct, peer-reviewed research. The NPG portfolio consists of *Nature Magazine*, its associated primary research journals, *Nature Reviews* journals, scientific and medical journals, and reference titles. *Nature Medicine* is a monthly international journal of biomedical research. *Nature Medicine* presents research that bridges the gap between cutting-edge biological research and more clinically orientated human investigations.

#### Nature Technology Corporation

4701 Innovation Dr.  
Lincoln, NE 68521  
Phone: 402-472-6530  
Fax: 402-472-6532  
E-Mail: [hodgson@natx.com](mailto:hodgson@natx.com)  
[www.natx.com](http://www.natx.com)

#### Booth Number: 203

Nature Technology Corporation (NTC) is dedicated to providing vector core services (design, construction and production – viral and non-viral) to researchers in the biopharmaceutical industry and academia. Acting as a 'virtual biotech company' to the industry, NTC offers state of the art molecular facilities, manufacturing support, testing and consulting.

#### Office of Biotechnology Activities

##### National Institutes of Health

6705 Rockledge Drive  
Suite 750  
Bethesda, MD 20892  
Phone: 301-496-9838  
Fax: 301-496-9839  
[www4.od.nih.gov/oba](http://www4.od.nih.gov/oba)

#### Booth Numbers: 205 & 207

The Office of Biotechnology Activities (OBA) at the National Institutes of Health will be exhibiting its new initiatives, and staff will be available to answer questions about the oversight of recombinant DNA research, including human gene transfer research. Meeting participants will be given "hands-on" demonstrations of OBA's new Genetic Modification Clinical Research Information System (GeMCRIS), as well as an extensive, new Web-based resource on informed consent in gene transfer research.

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Exhibitor Information

#### Olympus America Inc.

Two Corporate Center Drive  
Melville, NY 11747  
Phone: 631-844-5055  
Fax: 631-844-5111  
E-Mail: [olympusseg@econnexions.com](mailto:olympusseg@econnexions.com)  
[www.olympusamerica.com](http://www.olympusamerica.com)

#### Booth Number: 204

Olympus features the BX61 motorized research microscope with fluorescence and multi-wavelength FISH. Also on display is the BX51 fluorescence research microscope and the color CC12 FireWire™ digital camera. Olympus MicroSuite™ imaging software for acquisition, measurement, and analysis of data will also be available.

#### Omnia Biologics, Inc.

12115 D. Parklawn Drive  
Rockville, MD 20852  
Phone: 301-984-5928  
Fax: 301-984-2515  
E-Mail: [info@omniabiologics.com](mailto:info@omniabiologics.com)  
[www.omniabiologics.com](http://www.omniabiologics.com)

#### Booth Number: 405

Omnia Biologics provides cGMP manufacturing, filling, and process development services for gene therapeutics and vaccines. Our BSL-3 GMP facilities enable us to handle organisms that require a higher level of containment, such as oncolytic vectors and live vaccines. Custom services focus on the production and regulatory needs of small customers.

#### Open Biosystems

6705 Odyssey Dr.  
Huntsville, AL 35806  
Phone: 888-412-2225  
Fax: 256-704-4849  
E-Mail: [info@openbiosystems.com](mailto:info@openbiosystems.com)  
[www.openbiosystems.com](http://www.openbiosystems.com)

#### Booth Number: 417

Open Biosystems provides resources to unlock the functions of human genes and their relationships to disease development. Offering a wide range of cDNA clones from ORFs to ESTs, along with custom antibody services and high throughput custom peptide production, Open Biosystems brings superior quality and service to you.

#### Pall Life Sciences

25 Harbor Park Dr.  
Port Washington, NY 11050  
Phone: 516-801-9197  
Fax: 516-484-3877  
[www.pall.com](http://www.pall.com)

#### Booth Number: 504

#### PlasmidFactory GmbH & Co. KG

Meisenstr. 96  
Bielefeld D-33607  
Germany  
Phone: 49-527-299-7350  
Fax: 49-527-299-7355  
E-Mail: [info@PlasmidFactory.com](mailto:info@PlasmidFactory.com)  
[www.plasmidfactory.com](http://www.plasmidfactory.com)

#### Booth Number: 322

##### PlasmidFactory services:

- Endotoxin-free manufacturing of plasmid DNA in *Research Grade* quality
- Animal-free *ccc-Pilot Grade* DNA production including removal of inactive topologies and bacterial genomic DNA
- GMP contract manufacturing of plasmid DNA vectors
- CGE service analysis of customer plasmid samples
- GMP DNA storage service (ICH compliant)

#### ProMetic BioSciences, Inc.

155 Willowbrook Blvd., 4th Floor  
Wayne, NJ 07470  
Phone: 973-812-9880  
Fax: 973-812-9881  
[www.prometic.com](http://www.prometic.com)

#### Booth Number: 514

ProMetic BioSciences is a leading supplier of affinity purification solutions for research, development, and manufacturing in the biotechnology industry. ProMetic bases its products on proprietary synthetic ligand technology, resulting in a wide range of unique chromatography adsorbents for large and small scale applications including purification of antibodies, plasma proteins, plasmid DNA, transgenic therapeutics, enzymes, and biocontaminant removal.

# The American Society of Gene Therapy's 7th Annual Meeting

## final program

# 2004

### Exhibitor Information

#### Protech International, Inc.

405 N. St. Mary's, Suite 900  
San Antonio, TX 78205  
Phone: 210-229-1002  
Fax: 210-229-1003  
E-Mail: dennis@protechinternational.com  
[www.protechinternational.com](http://www.protechinternational.com)

#### Booth Number: 406

Protech International is an international distributor of analytical equipment. We are pleased to offer the CUY21EDIT Square Wave Electroporator, the Sonitron 2000 Sonoporation Unit, the iWorx Data Acquisition System, and a large inventory of EP Electrodes. See our online catalog at [www.protechinternational.com](http://www.protechinternational.com) for a complete line of equipment and supplies for gene therapy research.

#### Puresyn, Inc.

87 Great Valley Parkway  
Malvern, PA 19355  
Phone: 610-640-0800  
Fax: 610-640-0808  
E-Mail: purification@puresyn.com  
[www.puresyn.com](http://www.puresyn.com)

#### Booth Number: 311

Puresyn is a Separations and Purification Technology Company that develops, manufactures and markets state-of-the-art products and services for the separation and purification of nucleic acids and other biological molecules utilizing its proprietary polymer technology, PolyFlo®, an inert, irregularly shaped, non-porous polymer resin. The Company is a leading supplier of contract manufacturing services for production and purification of plasmid and viral vector-based gene therapies at scales and specifications suitable for research, development and pre-clinical requirements. Puresyn has also introduced two (2) new Adenovirus purification kits, ADENOPURE® and ADENOPURE LS™. They replace time and labor intensive double cesium chloride gradient and generate adenovirus preparations with high purity and potency in a convenient kit format.

#### Qbiogene, Inc.

2251 Rutherford Rd.  
Carlsbad, CA 92008  
Phone: 800-424-6101  
Fax: 760-918-9313  
E-Mail: info@qbiogene.com  
[www.qbiogene.com](http://www.qbiogene.com)

#### Booth Number: 312

Qbiogene supplies reagents and kits to the international research community. BIO101® Systems manufactures media and purification kits and Qbiogene Molecular Biology markets PCR, transfection, cloning, expression products, and FISH probes.

#### QIAGEN Inc.

28159 Avenue Stanford  
Valencia, CA 91355  
Phone: 800-426-8157  
Fax: 800-718-2056  
[www.qiagen.com](http://www.qiagen.com)

#### Booth Number: 517

The new QIAGEN Plasmid Prep Service provides milligram to gram amounts of high quality DNA in a fast turnaround time, for all applications in gene-therapy research and preclinical applications. With our pAlliance partners, QIAGEN offers a manufacturing service for cGMP-grade plasmid DNA for clinical through to commercial demands.

#### R&D Systems, Inc.

614 McKinley Pl. NE  
Minneapolis, MN 55413  
Phone: 612-379-2956  
Fax: 612-379-6580  
E-Mail: info@rndsystems.com  
[www.rndsystems.com](http://www.rndsystems.com)

#### Booth Numbers: 522

R&D Systems provides over 6,000 cytokine-related research products including recombinant and natural proteins; antibodies; kits and reagents for ELISAs, ELISpots, multiplex assays, flow cytometry, mRNA quantitation, stem cells, signal transduction, apoptosis, DNA damage and repair, cell selection, and cell culture; as well as primer pairs and assay services.

# The American Society of Gene Therapy's 7th Annual Meeting final program

# 2004

## Exhibitor Information

### Sangamo BioSciences, Inc.

501 Canal Blvd.  
Ste. A100  
Richmond, CA 94804  
Phone: 510-970-6000  
Fax: 510-236-8951  
E-Mail: erhodes@sangamo.com  
[www.sangamo.com](http://www.sangamo.com)

#### Booth Numbers: 511 & 513

Sangamo Biosciences is focused on the research and development of zinc finger DNA binding proteins (ZFPs) for therapeutic gene regulation and repair. The company's most advanced therapeutic program is for treatment of peripheral artery disease. Sangamo has additional programs in cardiovascular disease, cancer, neuropathic pain, and monogenic diseases.

### Sartorius BBI Systems Inc.

991 Postal Road  
Allentown, PA 18109  
Phone: 610-266-6262  
Fax: 610-266-6262  
E-Mail: michele.pavlinsky@sartorius.com  
[sartorius-bbi-systems.com](http://sartorius-bbi-systems.com)

#### Booth Number: 307

Sartorius BBI Systems is the leading supplier of fermentation and cell culture equipment—benchtop to production scale—worldwide. Laboratory scale product offering includes spinner flask and stirred tank bioreactors for suspension or attachment dependant cells. New products include the Medistat® tissue and cell culture system for gene-therapy and medical applications.

### SeqWright, Inc.

2575 West Bellfort, Suite 2001  
Houston, TX 77054  
Phone: 713-528-4363  
Fax: 713-528-6232  
E-Mail: seq@seqwright.com  
[www.seqwright.com](http://www.seqwright.com)

#### Booth Number: 415

SeqWright is a molecular biology research support company specializing in DNA discovery products and customized services designed for scientific researchers from the pharmaceutical, agricultural, biotechnology, government, and academic communities. We offer outsourcing options including de novo DNA sequencing and re-sequencing, DNA library construction, genotyping, quantitative PCR, and DNA synthesis.

### Southern Research Institute

2000 9th Avenue South  
Birmingham, AL 35205  
Phone: 205-581-2684  
Fax: 205-322-7473  
E-Mail: [brazier@sri.org](mailto:brazier@sri.org)  
[www.southernresearch.org](http://www.southernresearch.org)

#### Booth Number: 411 & 413

Southern Research is a science-driven, client-focused contract research organization involved in drug discovery and nonclinical drug development.

- Extensive experience in gene therapy product development (vector construction, efficacy evaluations, non-clinical toxicology, and biodistribution studies with Q-PCR)
- One of two National Toxicology Centers designated by the National Gene Vector Laboratories.

### St. Jude Children's Research Hospital

332 N. Lauderdale, MS 340  
Memphis, TN 38105  
Phone: 901-495-3424  
Fax: 901-495-5376  
E-Mail: [carl.jackson@stjude.org](mailto:carl.jackson@stjude.org)  
[www.stjude.org](http://www.stjude.org)

#### Booth Number: 524

### St. Louis Convention & Visitors Commission

One Metropolitan Square, Suite 1100  
St. Louis, MO 63102  
Phone: 800-325-7962  
Fax: 314-621-3470  
[www.explorestlouis.com](http://www.explorestlouis.com)

#### Booth: 313

The St. Louis Convention & Visitors Commission represents this historic, centrally located Mississippi River destination where you'll find the 502,000-square-foot America's Center convention complex, 33,000 hotel rooms, an amazing music and nightlife scene and more than a thousand one-of-a-kind restaurants.

# The American Society of Gene Therapy's 7th Annual Meeting final program

# 2004

## Exhibitor Information

### The Williamsburg BioProcessing Foundation

1206 Laskin Road, Suite 201

Virginia Beach, VA 23451

Phone: 757-423-8823

Fax: 757-423-2065

E-Mail: [cburba@wilbio.com](mailto:cburba@wilbio.com)

[www.wilbio.com](http://www.wilbio.com)

#### Booth Number: 208

The Williamsburg BioProcessing Foundation (WilBio) was established in 1994 to advance the development of safe and effective biological products by providing venues for the exchange of bioprocess technology. In 2002, WilBio launched the *BioProcessing Journal™* to enhance the exchange of bioprocess technology. Now in our 10<sup>th</sup> year, we have grown from six to 10 conferences and from one to three publications.

### Virapur, LLC

10461 Roselle Street

San Diego, CA 92121

Phone: 858-824-9000

Fax: 858-824-9408

E-Mail: [virapur@sbcglobal.net](mailto:virapur@sbcglobal.net)

[www.virapur.com](http://www.virapur.com)

#### Booth Number: 418

Virapur is a leader in developing virus purification kits and providing services to research and pharmaceutical communities. We directly market our Adenovirus Mini-Purification kits and the first ever AAV Purification kits for rapid virus purification without ultracentrifugation, while BD Clontech distributes our larger adenovirus purification kits. We also specialize in virus production, assays, process development, and consulting services.

### Waisman Clinical BioManufacturing Facility

1500 Highland Ave.

Madison, WI 53705

Phone: 608-263-8940

Fax: 608-263-5725

E-Mail: [Kench@waisman.wisc.edu](mailto:Kench@waisman.wisc.edu)

[www.waisman.wisc.edu/wcbf](http://www.waisman.wisc.edu/wcbf)

#### Booth Number: 324

The Waisman Clinical BioManufacturing Facility at the University of Wisconsin-Madison is academia's most versatile site for biologics and gene therapeutics production. WCBF is comprised of segregated production suites for viral, non-viral, and cell-based biologics, including a fill suite. Support labs include cell bank and quality laboratories. Visit us at [www.waisman.wisc.edu/wcbf](http://www.waisman.wisc.edu/wcbf) for more information.

### Wiley

111 River Street

Hoboken, NJ 07030

Phone: 201-748-6758

Fax: 201-748-6617

E-Mail: [rbenner@wiley.com](mailto:rbenner@wiley.com)

[www.wiley.com](http://www.wiley.com)

#### Booth Number: 404

Wiley is a global publisher of professional, consumer, scientific and technical books, journals, textbooks and education materials. Among its many imprints are the world renowned "For Dummies" line as well as Webster's New World, CliffNotes, Frommers, Betty Crocker books, Jossey-Bass and Wrox. To see more about these and our other imprints, visit Wiley at [www.wiley.com](http://www.wiley.com).

The American Society of Gene Therapy's 7th Annual Meeting  
final program

2004

**Notes**

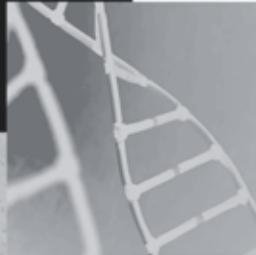


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THE DATE

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The American Society of Gene Therapy  
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June 1-5, 2005

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America's Center

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SAINT LOUIS

Watch for the "Call for Abstracts" this coming Fall!



**American Society of Gene Therapy**  
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E-Mail: [info@asgt.org](mailto:info@asgt.org)  
[www.asgt.org](http://www.asgt.org)

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