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Dear Colleagues,

On behalf of the American Society of Gene Therapy, welcome to the ASGT 8th Annual Meeting. We are very pleased to present this year’s program featuring the George Stamatoyannopoulos lecture by Thomas Tuschi, PhD, *Mechanisms of siRNA- and miRNA-guided Gene Regulation in Mammals*; the Presidential Symposium presentation by George Q. Daley, MD, PhD, *Combining Gene Therapy with Cell Therapy*; 18 Education Sessions; 16 Scientific Symposia; 17 Workshops; oral and poster sessions chosen from submitted abstracts and Meet-the-Investigator lunch sessions. This year, in response to feedback from previous years, the Young Investigators’ Symposium has been scheduled as a general session.

The Society’s committees have done a great deal of hard work in putting together this superb program and for that, I thank them. Topics that will be covered at this year’s meeting include: Cancer Vaccines, Pre-Clinical Issues in Gene Therapy, Stem Cell Gene Therapy, Gene Therapy and Behavior, Skin Gene Therapy, Gene Therapy for HIV, Ethical Issues in Clinical Trial Design, Nonviral Gene Delivery, and Regenerative Approaches for Skeletal Disorders. Many other sessions cover the diverse and exciting developments in the field over the past year.

I would like to thank our exhibitors and industry supporters for their participation and generosity, in addition to our exceptional faculty who kindly share their knowledge, expertise and time.

The ASGT Annual Meeting provides an outstanding forum for sharing the latest developments in the field of gene therapy and networking with our colleagues. I welcome you to St. Louis.

Sincerely,

Katherine A. High, MD
President, ASGT

ASGT EXECUTIVE OFFICE

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COMMITTEE MEETINGS

Committee terms technically commence and conclude in June at the Annual Meeting. Incoming Chair and committee member terms officially go into effect on June 3, 2005 at the Business Meeting.

<table>
<thead>
<tr>
<th>Wednesday, June 1</th>
<th>7:00 pm - 8:15 pm</th>
<th>2006 Planning Committee Meeting</th>
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<tr>
<td>Thursday, June 2</td>
<td>12:45 pm - 1:45 pm</td>
<td>Publications Committee</td>
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<td>12:45 pm - 1:45 pm</td>
<td>Ethics Committee</td>
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<td>12:45 pm - 1:45 pm</td>
<td>Education</td>
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<td>4:30 pm - 5:30 pm</td>
<td>Molecular Therapy Editorial Board</td>
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<tr>
<td>Friday, June 3</td>
<td>7:00 am - 8:00 am</td>
<td>Industrial Liaison Committee Meeting</td>
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<td></td>
<td>7:00 am - 8:00 am</td>
<td>Immunology of Gene Therapy</td>
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<td>7:00 am - 8:00 am</td>
<td>Oligonucleotide Based Therapies</td>
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<td>12:30 pm - 1:45 pm</td>
<td>Clinical &amp; Regulatory Affairs Committee</td>
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<td>Genetic Diseases</td>
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<td>Cancer Gene Therapy</td>
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<td>Nonviral Gene Transfer Vectors</td>
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<td>12:30 pm - 1:30 pm</td>
<td>Hemopoietic Cell Gene Therapy</td>
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<td>12:30 pm - 1:30 pm</td>
<td>Embryonic Stem Cells &amp; Tissue Engineering</td>
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<td>12:30 pm - 1:30 pm</td>
<td>Neural Disorders Gene Therapy</td>
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<td>12:30 pm - 1:30 pm</td>
<td>Infectious Disease</td>
</tr>
<tr>
<td>Saturday, June 4</td>
<td>12:15 pm - 1:30 pm</td>
<td>Advisory Board Meeting</td>
</tr>
<tr>
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<td>12:30 pm - 1:30 pm</td>
<td>Membership Committee</td>
</tr>
</tbody>
</table>

SOCIETY ANNUAL BUSINESS MEETING
The ASGT Business Meeting will take place in Room 130 on Friday, June 3, from 4:30 to 5:30 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.
COMMITTEE ROSTER  (As of April 27, 2005)

Terms begin and end at the June 8th Annual Business Meeting (through June 2005)

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For a complete schedule of Committee Meetings, see page 3
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PARTNER ($26,000+)

- Cell Genesys
  - Abstracts-on-Disc™
- Genzyme
  - Printing of Abstract Supplement
- Targeted Genetics
  - Registration Bags

CONTRIBUTOR ($10,000 - $25,000)

- March of Dimes
  - General Meeting Support
  - Supported in part by March of Dimes Birth Defects Foundation Grant No. 4-FY04-159
- NGVL
  - General Meeting Support

PATRON ($500 - $9,000)

- Avigen
  - Water Bottles
- Biogen Idec
  - General Meeting Support
- Ceregene
  - Three Travel Grants
- Fanconi Anemia Research Fund, Inc.
  - Excellence in Research Award
- GeneVec
  - General Meeting Support
- Introgen Therapeutics, Inc.
  - Three Excellence in Research Awards
- Mirus
  - Conference Notebooks
- National Heart, Lung and Blood Institute
  - General Meeting Support
- Sigma-Aldrich
  - Lanyards
- Sangamo
  - Three Excellence in Research Awards
  - Four Travel Grants
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Alzet® Osmotic Pumps, Durect Corporation
amaxa GmbH
American Society of Gene Therapy
Arkios BioDevelopment International
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Vivascience, Inc.
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Wiley
Williamsburg BioProcessing

EXHIBIT HALL HOURS

Thursday, June 2  2:00 pm – 7:30 pm
(Welcome Reception begins at 4:00 pm)

Friday, June 3  2:00 pm – 7:30 pm
(Networking Reception begins at 4:00 pm)

Saturday, June 4  3:00 pm – 7:00 pm
(Networking Reception begins at 4:00 pm)
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**RESEARCH AWARDS AND TRAVEL GRANT RECIPIENTS**

### EXCELLENCE IN RESEARCH AWARDS
**For Students and Postdoctoral Fellows**

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<td>Postdoctoral Fellow</td>
<td>Care of Established Disease and Direct Evidence of Cellular Cross-Correction in the Nervous System of Metachromatic Leukodystrophy Mice after HSC-Based Gene Therapy</td>
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<td>Jaeseok Han</td>
<td>Student</td>
<td>Glucose-Dependent Insulin Production by Liver-Specific, Glucose-Regulatable Synthetic Promoters Results in the Cure of Diabetes</td>
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<td>Katherine Bowman</td>
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<td>Ruth Seggewiss</td>
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<td>Yan Shou</td>
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<tr>
<td>Aurelie Goyenvalle</td>
<td>Student</td>
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FACULTY LIST

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GENERAL MEETING INFORMATION

AMERICAN SOCIETY OF GENE THERAPY

MISSION AND GOALS
The American Society of Gene Therapy (ASGT) is a professional non-profit medical and scientific organization dedicated to the understanding, development and application of gene and related cell and nucleic acid therapies and the promotion of professional and public education in the field.

ASGT is the largest medical professional organization representing researchers and scientists dedicated to discovering new gene therapies. ASGT was established in 1996, and has grown to close to 3,000 members from around the world. ASGT is committed to:

· Promoting Research, Development and Application
· Exchanging Information and Promote Education Among Professionals and the Public
· Promoting Development of Clinical Translations

The ASGT 8th Annual Meeting will serve as a comprehensive forum to achieve these goals.

NEEDS
Clinical gene transfer has become more and more 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. Oligonucleotide Based Therapies, RNAi and Vaccine Therapies will also be discussed as well as many other topics. This meeting will provide an educational forum for scientists and clinicians to expand their knowledge about the broad developments in these fields.

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/Video Presentations, Abstracts, Posters, Executive Summary.

TARGET AUDIENCE
The target audience includes basic science and translational researchers, clinical investigators, physicians, industry executives and scientists, postdoctoral fellows, graduate students, employees of federal government and regulatory agencies, 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. 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 or dropped in the evaluation collection boxes located throughout America’s 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 Stanford University School of Medicine and the American Society of Gene Therapy. Stanford University School of Medicine is accredited by the ACCME to provide continuing medical education for physicians.

Stanford University School of Medicine designates this educational activity for a maximum of 31 category 1 credits toward the AMA Physician’s Recognition Award. Each physician should claim only those hours of credit that he/she actually spent in the activity.

DISCLOSURE
It is the policy of Stanford University 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. Stanford University 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.

CME CERTIFICATES
CME Certificates will be available at the CME desk for participants requiring CME credit for this meeting.
GENERAL MEETING INFORMATION

CME SIGN IN
All participants requiring CME credit must sign in each day they are in attendance at the ASGT 8th Annual Meeting. The sign-in area is adjacent to the registration desk, outside of Hall 1 (Washington Avenue Lobby). A CME Certificate will not be available unless you sign in each day at the meeting.

ABSTRACT VOLUME
All abstracts accepted for presentation at the ASGT 8th 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 St. Louis.

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. Tickets will be collected at the door by ushers. There are still a limited number of tickets available which may be obtained at the Registration Desk. Tickets are $20 and include a box lunch.

BUSINESS CENTERS
Business centers are available for attendees at America’s Center and the Renaissance Grand. Please stop by either of the centers for a complete list of fees, services and hours.

BUSINESS MEETING
Friday, June 3, 2005
4:30 p.m. - 5:30 p.m.
Room 130
All members welcome!
See page 3 for more details.

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 8th Annual Meeting.

CELL PHONES
Please insure that cell phone ringers and pagers are turned off during all sessions.

NO SMOKING
Smoking is prohibited at all 8th Annual Meeting sessions and events.

JOB BANK
All job openings posted as of May 20, 2005 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 near the Registration desk outside of Hall 1 (Washington Avenue Lobby). 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.

INTERNET KIOSK POLICY
Internet access will be available to meeting attendees in two locations. One kiosk is located on Level One in the Plaza Lobby and the second kiosk is in the Second Floor Atrium on Level Two. Please limit your Internet use to 15 minutes at a time so that other attendees can access the Internet as well.

SHUTTLE BUS
ASGT will not be providing shuttle transportation for the 8th Annual Meeting in St. Louis. America’s Center is within a one block radius of the ASGT Annual Meeting hotels.

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. The ASGT staff will be happy to assist you with your specific needs.
GENERAL MEETING INFORMATION

EXHIBITS
Exhibits will be located in Hall 1 (Level One) of America’s 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 or registration area or in any location of America’s Center outside the rented exhibit space.

A Welcome Reception will be held in the Exhibit Hall on Thursday, June 2, and Networking Receptions in the Exhibit Hall on Friday, June 3, and Saturday, June 4. Receptions will begin at 4:00 pm daily.

EXHIBIT HALL HOURS
- Thursday, June 2: 2:00 pm – 7:30 pm
- Friday, June 3: 2:00 pm – 7:30 pm
- Saturday, June 4: 3:00 pm – 7:00 pm

LEAD RETRIEVAL
A lead retrieval system has been made available to all exhibitors of the ASGT 8th 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 2.

THURSDAY, JUNE 2
ABSTRACT POSTER SESSION I
- 10:00 am – Noon: Poster Setup by Authors
- Noon – 7:30 pm: Poster Viewing (authors present from 4:00 pm – 7:30 pm)
- 2:00 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 3
ABSTRACT POSTER SESSION II
- 7:00 am – Noon: Poster Setup by Authors
- Noon – 7:30 pm: Poster Viewing (authors present from 4:00 pm – 7:30 pm)
- 2:00 pm – 7:30 pm: Exhibit Hall Open
- 4:00 pm – 7:30 pm: Networking Reception
- 7:30 pm – 8:00 pm: Authors Remove Posters

SATURDAY, JUNE 4
ABSTRACT POSTER SESSION III
- 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 America’s Center, Level Two, Room 280.

Members of the working media may register for the 8th 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 1: 3:00 pm – 6:00 pm
- Thursday, June 2: 7:45 am – 5:00 pm
- Friday, June 3: 7:45 am – 5:00 pm
- Saturday, June 4: 7:45 am – 5:00 pm
- Sunday, June 5: 8:00 am – Noon

REGISTRATION DESK
Name badges, final programs, and abstract supplements will be distributed at the registration desk located on Level One of America’s Center, outside of Hall 1 (Washington Avenue Lobby).

REGISTRATION DESK HOURS
- Wednesday, June 1: Noon – 8:00 pm
- Thursday, June 2: 7:00 am – 5:00 pm
- Friday, June 3: 7:30 am – 5:00 pm
- Saturday, June 4: 7:30 am – 5:00 pm
- Sunday, June 5: 8:00 am – Noon

RESTAURANT INFORMATION DESK
St. Louis 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 St. Louis Convention & Visitors Commission, to help you make the right dining choice. Staff will have sample menus from area restaurants and will be able to assist you in making your reservations. The Restaurant Information Desk is located on Level One of America’s Center near Registration, outside of Hall 1 (Washington Avenue Lobby).
GENERAL MEETING INFORMATION

SPEAKER READY ROOM
The Speaker Ready Room is located in Room D (Level One) of America’s Center.

Important Notice: All faculty must deliver their presentations to the Speaker Ready Room the day before their session or at least five hours prior to their presentations. Equipment is available for faculty to review their materials. Audiovisual personnel will be available for assistance. Please mark your materials (include name, session and speaker order) so the information can be returned to you.

The Society strongly encourages faculty to pre-load presentations in the Speaker Ready Room; those faculty that load presentations during the session will have that time deducted from their presentation time by the Chair or Moderator.

SPEAKER READY ROOM HOURS

<table>
<thead>
<tr>
<th>Day</th>
<th>Hours</th>
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<tbody>
<tr>
<td>Wednesday, June 1</td>
<td>10:00 am – 9:00 pm</td>
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<tr>
<td>Thursday, June 2</td>
<td>6:30 am – 7:00 pm</td>
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<tr>
<td>Friday, June 3</td>
<td>7:00 am – 7:00 pm</td>
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<tr>
<td>Saturday, June 4</td>
<td>7:00 am – 6:00 pm</td>
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<tr>
<td>Sunday, June 5</td>
<td>7:30 am – 12:00 noon</td>
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</table>
## 2005 PROGRAM-AT-A-GLANCE

### WEDNESDAY, JUNE 1

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>8:00 am – 3:00 pm</td>
<td>Pre-Meeting Session: 2nd Stem Cell Clonality &amp; Genotoxicity Retreat</td>
</tr>
<tr>
<td>Noon – 8:00 pm</td>
<td>Attendee Registration Open</td>
</tr>
<tr>
<td>12:30 pm – 4:00 pm</td>
<td>ASGT Board of Directors Meeting</td>
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<tr>
<td>3:30 pm – 5:00 pm</td>
<td>Education Sessions I</td>
</tr>
<tr>
<td>5:15 pm – 6:45 pm</td>
<td>Education Sessions II</td>
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<tr>
<td>6:45 pm – 8:30 pm</td>
<td>Dinner Break</td>
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<tr>
<td>8:30 pm – 10:00 pm</td>
<td>Education Sessions III</td>
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### THURSDAY, JUNE 2

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>7:00 am – 5:00 pm</td>
<td>Attendee Registration Open</td>
</tr>
<tr>
<td>8:00 am – 9:00 am</td>
<td>George Stamatoyannopoulos Lecture and Excellence in Research Awards</td>
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<tr>
<td></td>
<td>Keynote Address: Thomas Tuschl, PhD</td>
</tr>
<tr>
<td>9:15 am – 12:30 pm</td>
<td>Workshops</td>
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<tr>
<td>10:00 am – Noon</td>
<td>Poster Setup by Authors</td>
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<tr>
<td>Noon – 7:30 pm</td>
<td>Poster Viewing</td>
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<tr>
<td>12:45 pm – 1:45 pm</td>
<td>Meet-the-Investigator Lunches</td>
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<tr>
<td>2:00 pm – 4:30 pm</td>
<td>Oral Abstract Sessions</td>
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<tr>
<td>2:00 pm – 7:30 pm</td>
<td>Exhibit Hall Open</td>
</tr>
<tr>
<td>4:00 pm – 7:30 pm</td>
<td>Poster Session, Exhibits, and Welcome Reception</td>
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### FRIDAY, JUNE 3

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<th>Time</th>
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<tr>
<td>7:00 am – Noon</td>
<td>Poster Setup by Authors</td>
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<tr>
<td>7:30 am – 5:00 pm</td>
<td>Attendee Registration Open</td>
</tr>
<tr>
<td>8:00 am – 10:00 am</td>
<td>Scientific Symposia</td>
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<tr>
<td>10:15 am – 12:15 pm</td>
<td>Scientific Symposia</td>
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<tr>
<td>Noon – 7:30 pm</td>
<td>Poster Viewing</td>
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<tr>
<td>12:30 pm – 1:30 pm</td>
<td>Meet-the-Investigator Lunches</td>
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<tr>
<td>2:00 pm – 4:30 pm</td>
<td>Oral Abstract Sessions</td>
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<tr>
<td>2:00 pm – 7:30 pm</td>
<td>Exhibit Hall Open</td>
</tr>
<tr>
<td>4:00 pm – 7:30 pm</td>
<td>Poster Session, Exhibits, and Networking Reception</td>
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<tr>
<td>4:30 pm – 5:30 pm</td>
<td>Business Meeting</td>
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<tr>
<td>8:30 pm – 10:00 pm</td>
<td>Evening Session: Immune Responses in Neurologic Gene Therapy</td>
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### SATURDAY, JUNE 4

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<th>Time</th>
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<td>7:00 am – Noon</td>
<td>Poster Setup by Authors</td>
</tr>
<tr>
<td>7:30 am – 5:00 pm</td>
<td>Attendee Registration Open</td>
</tr>
<tr>
<td>8:00 am – 10:00 am</td>
<td>Scientific Symposia</td>
</tr>
<tr>
<td>10:15 am – 12:15 pm</td>
<td>Oral Abstract Sessions</td>
</tr>
<tr>
<td>Noon – 7:00 pm</td>
<td>Poster Viewing</td>
</tr>
<tr>
<td>12:30 pm – 1:30 pm</td>
<td>Meet-the-Investigator Lunches</td>
</tr>
<tr>
<td>1:45 pm – 4:15 pm</td>
<td>Presidential Symposium and Presentation of Top Abstracts</td>
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<tr>
<td></td>
<td>Keynote Address: George Q. Daley, MD, PhD</td>
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<td>Presidential Address: Katherine A. High, MD</td>
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<tr>
<td>3:00 pm – 7:00 pm</td>
<td>Exhibit Hall Open</td>
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<tr>
<td>4:00 pm – 7:00 pm</td>
<td>Poster Session, Exhibits, and Networking Reception</td>
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<tr>
<td>4:30 pm – 6:00 pm</td>
<td>ASGT Board of Directors Meeting</td>
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</tbody>
</table>

### SUNDAY, JUNE 5

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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</thead>
<tbody>
<tr>
<td>8:00 am – Noon</td>
<td>Attendee Registration Open</td>
</tr>
<tr>
<td>8:30 am – 10:00 am</td>
<td>Young Investigators Symposium</td>
</tr>
<tr>
<td>10:15 am – 12:15 pm</td>
<td>Oral Abstract Sessions</td>
</tr>
<tr>
<td>12:15 pm</td>
<td>8th Annual Meeting Concludes</td>
</tr>
</tbody>
</table>
SCIENTIFIC PROGRAM

WEDNESDAY, JUNE 1, 2005

EDUCATION SESSION I – 3:30 PM - 5:00 PM

Education Session 100
Retrovirus and Lentivirus Vectors
This session will introduce three of the most popular versions of retroviral delivery systems, for which an increasing repertoire of strains and genera becomes available: gammaretroviral (based on murine leukemia virus), lentiviral (based on human immunodeficiency virus type 1) and spumaviral (based on “human” foamyvirus). They share the default aspects of the retroviral life cycle: receptor-mediated uptake of an enveloped particle, reverse transcription of a plus-stranded mRNA, and more or less random integration of double-stranded DNA following nuclear uptake of the preintegration complex. However, we will also discuss important differences in efficiency and biosafety related to viral origin and vector architecture.
Room: 263/267
Chair: Christopher Baum, MD
Speakers: Christopher Baum, MD - Retrovirus Vectors: We Are Family!
Luigi Naldini, MD, PhD - Lentiviral Vectors: Challenges and Expectations
David W. Russell, MD, PhD - Foamy Virus Vectors

Education Session 101
Nonviral Vectors
Nonviral gene therapy approaches may be simpler and safer than those using viral vectors. The challenges of non-viral gene therapy involve 1) development of vectors that provide long-term expression of therapeutic genes, and 2) development of suitable methods to deliver vector DNA to target cells. This session will examine the latest integrating and non-integrating vectors for achieving long-term gene expression and will describe current physical and chemical methods for DNA delivery.
Room: 260/264
Chair: Michele P Calos, PhD
Speakers: Michele P Calos, PhD - Integrating and Non-integrating Plasmids for Non-viral Gene Therapy
Daniel Scherman, PhD - In Vivo Electrotransfer of Plasmids or Synthetic Nucleotides: Basic Concepts and Potential Therapeutic Applications
Dexi Liu, PhD - What Constitutes a Better Synthetic Vector?

Education Session 102
Immune Responses
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.
Room: 120/124
Chair: Maria Grazia Roncarolo, MD
Speakers: Maria Grazia Roncarolo, MD - The Role of Regulatory T Cells in Suppressing Immune Responses to Transgenes and Inducing Immune Tolerance
Jacques Banchereau, PhD - Dendritic Cells: Controllers of Immune Responses

Education Session 103
Regulatory Hurdles to Phase III Clinical Trials
This session will discuss FDA's perspectives on what investigator/sponsors can do to help move the field of gene therapy forward and get products to licensure. FDA will discuss what is the most efficient way to establish safety and efficacy, as well as what type of preclinical studies should be performed prior to initiation of clinical trials. Questions regarding the conduct of exploratory and confirmatory clinical trials will be discussed. FDA will also discuss why product characterization is important during early product development. These issues will be discussed as a way of explaining how the academic investigator can play a role in partnering with industry to help bring gene therapy products to licensure.
Room: 265/266
Chair: Stephanie Simek, PhD
Speakers: Andrew Byrnes, PhD - Common Challenges in the Development of Gene Therapy Products
Mercedes Serabian, MS, DABT - Preclinical Considerations Beyond Phase 1
Daniel Rosenblum, MD - How to Make Optimal Use of the Approval Process
SCIENTIFIC PROGRAM

WEDNESDAY, JUNE 1, 2005

Education Session 104
Expression Cassette Design
Gene Therapy projects require expression of an exogenously introduced therapeutic gene. In The 1995 Orkin and Motulsky report identified inadequate knowledge of vector components necessary to maintain adequate expression of the therapeutic gene as a major fault in the field. Over the last 10 years, a significant amount of effort has been invested toward the development of optimized expression cassettes that provide sustained, high-level expression of therapeutic proteins in relevant disease models with viral and non-viral vectors. This session will provide guidance for the development of expression cassettes yielding high level, sustained expression of a desired therapeutic protein. The three presentations will provide (1) an overview to expression cassette design for viral and non-viral vectors, with a focus on using viral and house keeping promoters for highest level of gene expression initially following gene transfer, (2) strategies that have been successfully used to provide sustained, high level tissue-specific expression, and (3) vector strategies that optimize protein expression through the use of optimal intron configuration and mRNA splicing, mRNA stability elements and translation initiation site configuration. A question and answer session will follow.

Room: 123/127
Chair: Mitchell Finer, PhD
Speakers: Mitchell Finer, PhD - Expression Cassette Design - 20 Years in 20 Minutes
         Carol H. Miao, PhD - Establishment of High-Level and Tissue-Specific Gene Expression Cassettes
         Thomas J. Hope, PhD - Post Transcriptional Regulation in Gene Expression Vectors

Education Session 105
Cancer Gene Therapy
Cancer has been a target application for numerous gene therapy trials. Recent advances in the use of oncolytic viruses, the type of anticancer transgenes employed for biologic effects and the effects of the immune system will be discussed. The understanding of these topics will facilitate improved applications in human trials.

Room: 240 Complex
Chair: E. Antonio Chiocca, MD, PhD
Speakers: E. Antonio Chiocca, MD, PhD - HSV Oncolytic Viruses
          Glen Barber, PhD - VSV as an Oncolytic Vector: Mechanisms of Action
          Noriyuki Kasahara, MD, PhD - Retrovirus Vectors for Cancer Gene Therapy: Oncolytic, Anti-angiogenic, and Immunotherapeutic Strategies

BREAK – 5:00 PM - 5:15 PM

EDUCATION SESSION II – 5:15 PM - 6:45 PM

Education Session 110
Adenovirus Vectors
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 adenoviruses 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.

Room: 263/267
Chair: Philip L. Leopold, PhD
Speakers: Neil R. Hackett, PhD - Molecular Biology of Adenovirus Vectors
          Philip L. Leopold, PhD - Cell Biology of Adenovirus Infection: Subcellular Mimicry
          James M. Wilson, MD PhD - Immunology of Adenoviral Vectors

Education Session 111
HSV Vectors
This session will describe design features and production features, plus experimental and therapeutic uses of recombinant oncolytic, replication defective and amplicon vectors derived from herpes simplex virus type 1. Oncolytic vectors are designed for treatment of cancer based on targeted delivery and selective virus replication in tumor cells. Clinical trials using these vectors for brain tumors will be described. Replication defective viruses deleted for essential viral functions have been engineered for peripheral nervous system applications since they naturally persist in sensory nerves following local delivery to the skin. Amplicon vectors incorporate no viral genes and can include up to 150 kb of genetic information, including complete genes, self-contained regulatory elements and AAV genome integrating components. These vectors provide versatile tools for systematic genetics and therapeutics.

Room: 260/264
Chair: Xandra Breakefield, PhD
Speakers: Xandra Breakefield, PhD - Mega-multi Capacity HSV Amplicon Vectors
          Joseph C. Glorioso, PhD - HSV Gene Vectors for Treatment of Pain and Neuropathy
          E. Antonio Chiocca, MD, PhD - HSV Vectors for Chemotherapy Delivery
SCIENTIFIC PROGRAM

WEDNESDAY, JUNE 1, 2005

Education Session 112
Regulation of Transgene Expression
The ability to regulate the timing and level of transgene expression opened new avenues in basic research applications and might become prerequisites in future clinical settings. Several gene regulation systems have been developed and successfully incorporated into viral and non-viral gene delivery systems. The session will begin with a general overview on the characteristics common to the various gene expression regulating systems. The following parts of the session will focus on the most common gene regulatory systems including the tetracycline, ecdysone, and the dimerizer systems. The last part of this session will cover new developments in regulating siRNA expression cassettes.

Room: 265/266
Chair: Tal Kafri, PhD
Speakers: Tal Kafri, PhD - General overview on transgene regulation with special emphasis on the tetracycline gene regulatory system
Francesco Galimi, MD, PhD - Regulation of Transgene Expression: Vector Designs and Applications
Jakob Reiser, PhD - Knockdown Control: Practical Approaches to Regulating shRNA Expression

Education Session 113
NIH Grantsmanship & Funding Opportunities
The goal of this session is to acquaint participants with training and career development application mechanisms and the review process at the NIH. Staff will give advice on what contributes a successful grant application and how to pick the right mechanism depending on the applicant’s expertise and education. There will be an opportunity for questions during the presentations and NIH staff will be available for individual questions. This session is intended for new investigators but should be informative for any investigator planning to submit an NIH grant application.

Room: 120/124
Chair: Sonia I. Skarlatos, PhD
Speakers: Sonia I. Skarlatos, PhD - Research Training and Career Development Programs
Steven J. Zullo, PhD - Grant Review in the CSR
Arun Srivastava, PhD - Writing and Reviewing NIH Grants: A Personal Odyssey

Education Session 114
Gene Transfer to Muscle
This educational session will focus on applications, methods and strategies for gene transfer to muscle tissue. The first lecture will give an overview of viral vectors for muscle gene transfer in vivo. The advantages and disadvantages of a variety of commonly used vectors with examples of their application will be discussed. The second lecture will focus on non-viral methods for gene transfer to muscle, which is uniquely suited for uptake of non-viral vectors. The final topic will be on ex vivo methods for gene transfer and appropriate vectors for transducing stem cells.

Room: 123/127
Chair: Jeffrey Chamberlain, PhD
Speakers: Jeffrey Chamberlain, PhD - Viral Gene Transfer
Jon Wolff, MD - Non-Viral Gene Transfer
Giuliana Ferrari, PhD - Cell Therapy for Muscular Dystrophy

Education Session 115
Building a Strategy for Translational Medicine: Focus on Immunodeficiencies
The goal of this session is to highlight scientific, technological and logistical aspects that bear on the successful translation and clinical implementation of gene therapy research. This year’s session will focus on the severe combined immune deficiencies (SCID). The faculty will discuss their experience with adenosine deaminase (ADA) deficiency, X-linked SCID and Wiskott-Aldrich syndrome, with emphasis on the strategic choices they have made and the hurdles they have confronted on issues such as vector/promoter selection, small vs. large animal models, vector production, infrastructure development, protocol approval, patient selection and recruitment, quality assurance and regulatory oversight.

Room: 230/231
Chair: Michel Sadelain, MD PhD
Speakers: Donald B. Kohn, MD - ADA-Deficient SCID: Pathogenis and Treatments
Marina Cavazzana-Calvo, MD, PhD - Severe Combined Immunodeficiency: Disease Models for New Therapeutical Approaches
Arthur Nienhuis, MD - Development of Gene Therapy for Wiskott-Aldrich Syndrome

DINNER BREAK – 6:45 PM - 8:30 PM
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WEDNESDAY, JUNE 1, 2005

EDUCATION SESSION III – 8:30 PM - 10:00 PM

Education Session 120
Novel Vector Systems
Novel adenovirus and adeno-associated virus vectors have been developed recently, which enable the delivery of transgenes into target cells and tissues with efficiency and specificity. Application of these novel vectors in the delivery of therapeutic genes to treat inherited disorders as well as acquired diseases like cancer will be discussed.

Room: 260/264
Chair: Savio L.C. Woo, PhD
Speakers: Andre Lieber, MD, PhD - Gene Transfer Vectors Based on B-group Adenoviruses
David T. Curiel, MD, PhD - Targetable/Injectable Adenoviral Vectors for Cell-specific Gene Transfer In Vivo
Guangping Gao, PhD - Efficient Gene Transfer Vectors Based on Novel Primate AAVs

Education Session 121
AAV Vectors
The goal of the AAV Education Session is to provide the community not only with a general understanding of this popular vector system, but to integrate this into a broader awareness of expectations when using these vectors in vitro and in vivo. Three topics will be discussed in this session. The first speaker will begin with a general background of AAV biology including a discussion of the molecular fate of recombinant genomes in vitro and in vivo. The second topic will concentrate on structural differences between AAV serotypes and how those differences can be utilized to improve the vector delivery. The final topic will focus on applications of AAV serotype delivery to specific target organs including performance comparisons between different serotypes.

Room: 263/267
Chair: Joseph Rabinowitz, PhD
Speakers: Joseph Rabinowitz, PhD - Exploring the Structure and Function of the AAV Virion
Xiao Xiao, PhD - Recent Progress in AAV Vector Development
K. Reed Clark, PhD - Wild-type and Recombinant AAV Biology - An Update

Education Session 122
RNAi
RNA interference refers to a cell’s 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.

Room: 240 Complex
Chair: Beverly L. Davidson, PhD
Speakers: Beverly L. Davidson, PhD - RNA Interference
John J. Rossi, PhD - The Long and Short of RNAi
Michael McManus, PhD - Developmental and Post-developmental Phenotypes of MicroRNA Ablation in the Mouse

Education Session 123
Gene Transfer to the Central Nervous System
In this session the three speakers will address specific issues pertaining to gene transfer in the nervous system. The broad goals are to convey to the audience the anatomic, immunologic and development aspects of the nervous system that are unique as a target for gene therapy. Dr. Fink will highlight the peripheral nervous system development, anatomy, function and disease related dysfunctions. Dr. Maria Castro will address neural immune interactions, locus of antigen presentation and consequences for gene transfer. Dr. Federoff will touch on special problems encountered in the translation of preclinical gene therapy. He will also briefly highlight issues related to the development of in utero CNS gene therapy.

Room: 230/231
Chair: Howard J. Federoff, MD, PhD
Speakers: Howard J. Federoff, MD, PhD - Translational Considerations for CNS Gene Therapy
David J. Fink, MD - Gene Transfer to DRG for Treatment of Neuropathy or Chronic Pain
Maria Castro, PhD - Regulated, Long Term Therapeutic Gene Expression for Neurological Gene Therapy
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WEDNESDAY, JUNE 1, 2005

Education Session 124
Bioavailability and Pharmacokinetics
Pharmacokinetic analysis translates the disposition characteristics of macromolecules into quantitative parameters that can be used for direct comparison of medicinal agents as well as with physiological parameters such as blood flow and the rate of cellular endocytosis. Careful collection and experimental analysis of biological samples as well as theoretical development of appropriate mathematical models are necessary for accurate prediction of drug disposition and comparison of treatment regimens. This session, designed for the bench scientist and the clinician alike, will discuss basic pharmacokinetic concepts, methods for modeling, study design, data analysis, population kinetics and their application to gene delivery systems.

**Room:** 265/266

**Chair:** Maria A. Croyle, PhD

**Speakers:**
- Maria A. Croyle, PhD - Pharmacokinetics Made Easy
- Sally Choe, PhD - Bridging Preclinical and Clinical Pharmacokinetics in Gene Therapy

Education Session 125
Gene-Based Vaccines
This session will give the fundamentals as well as updates about Gene-based vaccines. The technology, immune mechanisms, and second generation advances describing new delivery systems and the use of adjuvants will be presented. An overview of clinical and research applications will be described.

**Room:** 120/124

**Chair:** Margaret A. Liu, MD

**Speakers:**
- Margaret A. Liu, MD - Gene-based Vaccines: An Overview
- Jeffrey Ulmer, PhD - Toward the Development of an Effective DNA Vaccine for Humans
- Hildegund CJ Ertl, MD - Adaptive Immune Responses to Viral Vectors

THURSDAY, JUNE 2, 2005

GEORGE STAMATOYANNOPOULOS LECTURE 200
8:00 am - 9:00 am

**Room:** America’s Ballroom

**Chair:** Mark A. Kay, MD, PhD

**Speaker:** Thomas Tuschl, PhD - Mechanisms of siRNA- and miRNA-guided Gene Regulation in Mammals

Presentation of Excellence in Research Awards

BREAK – 9:00 AM - 9:15 AM

WORKSHOPS – 9:15 AM - 10:45 AM

Workshop 210
Cancer: Optimization of T-Cell Gene Therapy

**Room:** 123/127

**Chair:** Laurence JN Cooper, MD, PhD

**Speakers:**
- John C. Morris, MD - Role of Interleukin-15 in T cell Development and Function
- Laurence JN Cooper, MD, PhD - Redirected Specificity of T cells - Improving Their Potential
- Steven M. Albelda, MD - Augmenting T-cell Trafficking to Tumors
- Bruce L. Levine, PhD - Cancer: Optimization of T-Cell Gene Therapy
- Francesco M. Marincola, MD - Gene Profiling of Immune Responses Against Tumors
- Carolyn Wilson, PhD - T-cell Gene Therapy: Regulatory Issues
Workshop 211
Gene-Based Vaccines: New Developments for Genetic Vaccines
Room: 120/124
Chair: David B. Weiner, PhD
Speakers: Richard Heller, PhD - Electroporation Mediated Immunotherapy of Solid Tumors: Preclinical Results and Initiation of Clinical Study
Leaf Huang, PhD - LPD Nanoparticles: A Gene Vector Turned into a Potent Vaccine Carrier
David B. Weiner, PhD - Expanding the Potency and Breadth of the Immune Responses Induced by DNA Vaccines
Britta Elisabeth Wahren, MD, PhD - Means to Induce Mucosal Immunity in HIV Genetic Immunization
Ning-Sun Yang, PhD - Developing Multiple Viral Protein Genes and Virus-like Particles as Gene-based Vaccines

Workshop 212
Hemopoietic: Hemopoietic Stem Cell Gene Therapy
Room: Ferrara Theater
Chair: Jan Nolta, PhD
Speakers: Carolyn Wilson, PhD - Addressing Safety Concerns for Hematopoietic Stem Cell Gene Therapy - A FDA Perspective
Manuel Grez, PhD - Correction of Chronic Granulomatous Disease by Gene Therapy
Marina Cavazzana-Calvo, MD, PhD - Clinical and Biological Results from the SCID-XI Gene Therapy Trial
Stanton L. Gerson, MD - In Vivo Stem Cell Selection without Myeloablation
Alessandro Aiuti, MD, PhD - Gene Therapy of ADA-deficient SCID

Workshop 213
Immunology of Gene Therapy: Organ Specific Immunity
Room: 260/264
Chair: Roland W. Herzog, PhD
Speakers: Pedro Lowenstein, MD, PhD - Immune Responses to Viral Vectors in the Central Nervous System
John Iacomini, PhD - Reshaping the Immune System Using Gene Therapy
Roland W. Herzog, PhD - In Vivo Gene Transfer to Liver and Muscle; Two Organs, Two Different Stories
Hans-Peter Kiem, MD - Immune Responses to Gene-modified Hematopoietic Cells

Workshop 214
Industrial Liaison: Pre-Clinical Issues in Gene Therapy
Room: 263/267
Chair: Douglas J. Jolly, PhD
Speakers: Rebecca Sheets, PhD - Pre-clinical Development of Adenovirus-vectored HIV Vaccine Candidate
Joy Cavagnaro, PhD, DABT, RAC - Optimizing Design and Analysis of Preclinical Development Programs
Boro Dropulic, PhD - Clinical Translation of a Lentiviral Vector for the Treatment of HIV/AIDS
Peter K. Working, PhD, DABT - Preclinical Strategies: Taking an "Armed" Oncolytic Adenovirus to the Clinic

Workshop 215
Programs of Excellence in Gene Therapy
Room: 230/231
Chair: Sonia I. Skarlatos, PhD
Speakers: Sonia I. Skarlatos, PhD - PEGT Overview
Haig H. Kazazian, Jr., MD - Preclinical Gene Therapy of Hemophilia A with New AAV Serotypes
Ronald G. Crystal, MD - Gene therapy for Alpha 1-antitrypsin Deficiency with Intrapleural Administration of Novel AAV Serotypes
Barry London, MD, PhD - Gene Therapy During LVAD Support: Can We Fix the Broken Heart?
Stanley Riddell, MD - Regulating T cells for Immunotherapy by Gene Insertion
Richard A. Knazek, MD - The National Gene Vector Laboratories - Production, Toxicology, Repository, Clonality Testing and Long-Term Follow Up

Workshop 216
Room: 265/266
Chair: Stephen Hyde, PhD
Speakers: Bruce Bunnell, PhD - Mouse or Monkey: What is the FDA Looking for in Pre-clinical Data?
Alice F. Tarantal, PhD - Monkey Model for Fetal Intrapulmonary Gene Transfer
John F. Engelhardt, PhD - Are Men Mice or Pigs? A Lung Gene Therapy and Cell Biology Perspective
Uta Griesenbach, PhD - Fabp-CF Knockout Mice as Models for CFTR Gene Transfer
Gerry McLachlan, PhD - Evaluation Non-viral Gene Transfer in the Ovine Lung
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Workshop 217
Viral: Key Design Issues for Oncolytic Vectors
Room: 240 Complex
Chair: Samuel D. Rabkin, PhD
Speakers: David L. Bartlett, MD - Options for Creating Mutant Poxviruses for Oncolytic Therapy
Noriyuki Kasahara, MD, PhD - Building a Better Mouse Virus: Design Strategies to Improve Therapeutic Efficacy and Safety of Replication-competent Retrovirus (RCR) Vectors for Cancer Gene Therapy
John C. Bell, PhD - VSV as an Oncolytic Agent
Stephen J. Russell, MD, PhD - Retargeting Attachment and Entry of Oncolytic Viruses
William S. M. Wold, PhD - Oncolytic Adenovirus Vectors that Overexpress ADP
Samuel D. Rabkin, PhD - Oncolytic Herpes Simplex Virus (HSV) Vectors

BREAK – 10:45 AM - 11:00 AM

WORKSHOPS – 11:00 AM - 12:30 PM

Workshop 220
Cardiovascular Gene Therapy: From Bench to Bedside
Room: 120/124
Co-Chairs: Anthony Rosenzweig, MD
Roger J. Hajjar, MD
Speakers: Albert J. Sinusas, MD - Imaging of Angiogenesis and Gene Therapy In Vivo
R. Jude Samulski, PhD - Biological Nanoparticles: The Next Generation of Efficient Delivery Reagents
David Kaye, FRACP, PhD, FACC - Percutaneous Approaches to Cardiac Gene Transfer
Roger J. Hajjar, MD - The Road from Physiological Observation to Gene Therapy in Patients with Heart Failure

Workshop 221
Clinical and Regulatory Affairs: Cell and Gene Based Cancer Vaccines
Room: 263/267
Chair: Dale Ando, MD
Speakers: Karin Jooss, PhD - Combination Therapy of a GM-CSF Secreting Tumor Cell Vaccine and anti-CTLA-4: Preclinical Evaluation and Toxicology Studies
Drew Pardoll, MD, PhD - Targeting Immune Responses to Sites of Cancer Using Engineered Viruses and Bacteria
Stephen J. Russell, MD, PhD - Noninvasive Expression Monitoring of Oncolytic Viruses in Phase I Clinical Trials

Workshop 222
Genetic Diseases: Focus on the Metabolic Diseases of the Liver
Room: 265/266
Chair: Jayanta Roy-Chowdhury, MD
Speakers: Markus Grompe, MD - Strategies to Select Hepatocytes In Vivo
Cary Harding, MD - Gene and Cell-mediated Therapeutic Approaches to Phenylketonuria
Jayanta Roy-Chowdhury, MD - SV40-based Vectors for Metabolic Diseases of the Liver and Beyond
Brendan Lee, MD, PhD - Helper-dependent Adenoviral Gene Therapy for Metabolic Liver Diseases

Workshop 223
Infectious Disease: Innate Immunity and Vector Function
Room: 123/127
Co-Chairs: Bruce L. Levine, PhD
John A. Zaia, MD
Speakers: Hildegund CJ Ertl, MD - Innate Immunity to Adenovirus
Bryan Williams, PhD - Synergistic Activation of Innate Immunity by Double-stranded RNA and CpG DNA
Bruce E. Torbett, PhD, MSPH - Are Viral-based Vectors and Their Payloads Seen as Pathogens?
Hua Yu, PhD - Stat3 Targeting and Immunity
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Workshop 224
Musculo-Skeletal: Myogenic Stem Cells and Regeneration
Room: 230/231
Co-Chairs: Jeffrey Chamberlain, PhD
Shin’ichi Takeda, MD, PhD
Speakers: Shin’ichi Takeda, MD, PhD - Contribution of CD31-negative/CD45-negative Side Population Cells to Skeletal Muscle Regeneration
Morayma Reyes, MD, PhD - Muscle Multipotent Adult Progenitor Cells (MAPC): Their Origin, Role and Clinical Potential in Muscle Disease
Stephen D. Hauschka, PhD - Regulatory Cassette Design for Optimal Expression in Skeletal and Cardiac Muscle Following In Vivo and Ex Vivo Gene Delivery
Thomas A. Rando, MD, PhD - Molecular Control of Muscle Stem Cell Activity
Johnny Huard, PhD - Gene Therapy Based on Muscle-derived Stem Cells: Potential for Tissue Regeneration and Repair
Emanuela Gussoni, PhD - Human Muscle Progenitor Cell Quiescence Maintained by Elevated BMP4 Expression

Workshop 225
Neural Disorders: Gene Therapy and Behavior
Room: Ferrara Theater
Chair: Pedro Lowenstein, MD, PhD
Speakers: Mary Bartlett Bunge, PhD - Using Gene Therapy to Improve Repair of the Injured Spinal Cord
William J. Bowers, PhD - Functional Assessment of Gene-based Aβ Immunotherapy in a Mouse Model of Alzheimer’s Disease
James B. Uney, PhD - Practical Approaches to Using Viral Vectors Provisional to Study the Molecular Basis of Behavioral Function
Michael Kaplitt, MD, PhD - Gene Therapy to Alter Sexual Behavior
Xandra Breakfield, PhD - HSV Amplicon Vector Delivery of Gonadotropin Releasing Hormone Gene (13 kb) to Hypothalamus Restores Estrous Activity in Hypogonadal Mice

Workshop 226
Nonviral: New Approaches in Nonviral Gene Delivery
Room: 260/264
Co-Chairs: Stephen Hart, PhD
Leaf Huang, PhD
Speakers: Esther H. Chang, PhD - Tumor Targeting Nanodelivery Systems: Expanding the Potential for Cancer Therapy and Diagnosis
Mark E. Davis, PhD - A Systems Approach to the Design and Development of Non-Viral Delivery Systems
Muna Naash, PhD - DNA Nanoparticles for Non-Viral Ocular Gene Transfer
Lonnie D. Shea, PhD - Controlled Release Systems for Non-viral Vectors
Alexander V. Kabanov, PhD - Transcriptional Activation of Gene Expression by Pluronic
Ian MacLachlan, PhD - Clinical Pharmacology of Stable Plasmid Lipid Particles for Systemic Administration

Workshop 227
Special: The Clinical Trial Data on adp53 Gene Therapy of Cancer
Room: 240 Complex
Co-Chairs: James S. Norris, PhD
Helen E. Heslop, MD
Speakers: Jack A. Roth, MD - Adp53 Clinical Trials
Dmitry Gabrilovich, MD, PhD - Immunotherapy of Cancer with Dendritic Cell Based p53 Vaccine
Yongsong Guan, MD - Transcatheter Hepatic Arterial Chemoembolization and Gendicine Combined in Treatment of Advanced Hepatocellular Carcinoma (HCC)

Workshop 228
Special: Skin Gene Therapy
Room: 261/262
Chair: Ulrich R. Hengge, MD
Speaker: Ulrich R. Hengge, MD - Naked DNA: From Basics to Application
Ernst Wagner, PhD - Bioreponsive Deshielding of Targeted DNA Polyplexes
Lorne Taichman, MD, MSc, PhD - Long-term Systemic Delivery From Genetically Modified Human Skin

MEET-THE-INVESTIGATOR LUNCH
SESSIONS – 12:45 PM - 1:45 PM

Meet-the-Investigator 230
Room: Washington Avenue A
Chair: Jeffrey Chamberlain, PhD - Systemic Delivery of Genes to Muscle Using rAAV
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Meet-the-Investigator 231
Room: Washington Avenue B
Chair: Carl H. June, MD - Translational Lessons on T Cell Gene Transfer

Meet-the-Investigator 232
Room: Washington Avenue C
Chair: Donald B. Kohn, MD - Gene Therapy Using Hematopoietic Stem Cells

Meet-the-Investigator 233
Room: Washington Avenue E
Chair: Gerry McLachlan, PhD - Can Animal Models Predict Clinical Efficacy of Respiratory Gene Transfer?

Meet-the-Investigator 234
Room: Washington Avenue F
Chair: Maria Grazia Roncarolo, MD - Is Immune Response to Transgenes a Major Hurdle to Successful Gene Therapy?

SIMULTANEOUS ORAL ABSTRACT SESSIONS – 2:00 PM - 4:30 PM

Oral Abstract Session 240
RNA Virus Vectors: Gene Transfer and Gene Expression
Room: 260/264
Co-Chairs: Stephen J. Russell, MD, PhD
John Tisdale, MD
(Abstracts #1 - #10)

Oral Abstract Session 241
AAV Vectors: Vector Biology
Room: 240 Complex
Co-Chairs: Hiroyuki Nakai, MD, PhD
John F. Engelhardt, PhD
(Abstracts #11 - #20)

Oral Abstract Session 242
Adenovirus Vectors: Vector Technology
Room: Ferrara Theater
Co-Chairs: Kohnosuke Mitani, PhD
Michael J. Imperiale, PhD
(Abstracts #21 - #30)

Oral Abstract Session 243
Inborn Errors of Metabolism - Stem Cells and Blood Disorders
Room: 120/124
Co-Chairs: Barry J. Byrne, MD PhD
Mark S. Sands, PhD
(Abstracts #31 - #40)

Oral Abstract Session 244
Cardiac Gene Transfer
Room: 123/127
Chair: Federica Del Monte, MD, PhD
(Abstracts #41 - #50)

Oral Abstract Session 245
Neurodegenerative Diseases
Room: 263/267
Co-Chairs: David J. Fink, MD
Martha C. Bohn, PhD
(Abstracts #51 - #60)

Oral Abstract Session 246
Gene Therapy for Infections and Vaccines
Room: 230/231
Co-Chairs: Boro Dropulic, PhD
Bruce E. Torbett, PhD, MSPH
(Abstracts #61 - #68)

Oral Abstract Session 247
Cancer-Targeted Gene Therapy: Non Adenoviral Targeting of Vectors and Non Viral Vectors
Room: 265/266
Co-Chairs: Ronald G. Crystal, MD
Albert B. Deisseroth, MD, PhD
(Abstracts #69 - #78)

Oral Abstract Session 248
Gene Regulation: Keeping Genes On and Turning Them Off
Room: 261/262
Co-Chairs: Punam Malik, MD
Brian P. Sorrentino, MD
(Abstracts #79 - #88)

POSTER SESSION I – 4:00 PM - 7:30 PM

RNA Virus Vectors: Gene Expression
(Abstracts #89 through #109; and Abstract #822)

AAV Vectors: Disease Applications
(Abstracts #110 through #136)

Adenovirus Vectors: General Biology and Host Response
(Abstracts #137 through #154)

“Other” DNA Virus Vectors
(Abstracts #155 through #175)

Naked DNA Gene Transfer: Chromosomal, Integration, Delivery Techniques
(Abstracts #176 through #195)

Molecular Conjugates
(Abstracts #196 through #231)
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Inborn Errors of Metabolism: Systemic and Storage Diseases
(Abstracts #232 through #250)

Muscle and Connective Tissue: Gene Therapy for Muscular Dystrophy
(Abstracts #251 through #266)

Cancer - Immunotherapy: Genetically Modified Antigen Presenting Cells and Adjuncts
(Abstracts #267 through #285)

Cancer - Apoptosis and Suicide
(Abstracts #286 through #309)

Cancer - Targeted Gene Therapy: Targeting of Non-Viral Vectors
(Abstracts #310 through #329; Abstract # 328 has been moved to Poster Session III)

Hematologic - Disease Models
(Abstracts #330 through #349)

Gene Therapy Approaches to Pulmonary Disease
(Abstracts #350 through #367)

Gene Regulation: Gene Targeting and RNA
(Abstracts #368 through #385)

FRIDAY, JUNE 3, 2005

SCIENTIFIC SYMPOSIA – 8:00 AM - 10:00 AM

Scientific Symposium 300
Ethics: Ethical Issues in Clinical Trial Design:
Informed Consent and Children
Room: Ferrara Theater
Chair: R. Scott McIvor, PhD
Speakers:
Adrian J. Thrasher, MD, PhD - Clinical Trials of Gene Therapy for Severe Combined Immunodeficiency
Robert M. Nelson, MD, PhD - Gene Transfer Trials in Children: What’s Ethical? What’s Allowable?
Nancy M.P. King, JD - Children in Gene Transfer Trials: Design, Benefit and Consent Issues
Panel:
Punam Malik, MD
Cynthia Dunbar, MD

Scientific Symposium 301
Gene-Based Vaccines: Cancer Vaccines
Room: 230 Complex
Chair: Margaret A. Liu, MD
Speakers:
Kristen Hege, MD - GM-CSF Gene-modified Cancer Vaccines
Margaret A. Liu, MD - Clinical Gene-based Cancer Vaccines and Immunotherapy
Malcolm K. Brenner, MD, PhD - Improving the Function and Feasibility of Gene Modified Cancer Vaccines
Dennis Panicali, PhD - Progress in the Development of Poxvirus-based Cancer Vaccines

Scientific Symposium 302
Genetic Diseases: Protein Modifications to Enhance Therapy of Genetic Disease
Room: 240 Complex
Chair: Valder Arruda, MD, PhD
Speakers:
Jonathan B. Rothbard, PhD - Delivery of Small and Large Molecules into Cells and Tissues with Arginine Rich Transporters
Robert H. Costa, PhD - Use of a Transducing p19ARF Peptide to Induce Apoptosis and Diminish Proliferation of Mouse Hepatocellular Carcinomas
Valder Arruda, MD, PhD - Coagulation Proteins with Improved Biological Properties for Hemostasis Gene Therapy
Nay Wei Soong, PhD - Seven Years in Tibet: Optimizing Therapeutic Proteins by DNA Shuffling
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FRIDAY, JUNE 3, 2005

Scientific Symposium 303
Infectious Disease: Gene Therapy for HIV: Lessons Learned, Paradigms to Apply to Other Areas

Room: 260/264
Co-Chairs: Boro Dropulic, PhD
Ramesh Akkina, DVM, PhD
Speakers: Dale Ando, MD - Gene Modulation of the HIV Co-receptor CCR5 Using CCR5 Specific Zinc Finger Nucleases
Dorothy Von Laer, MD - Treatment of HIV Infection with Gene Protected Autologous T cells
Carl H. June, MD - Lentiviral Gene Therapy for HIV: A New Paradigm?
John J. Rossi, PhD - RNAi Gene Therapy for HIV-1

Scientific Symposium 304
Musculo-Skeletal: Regenerative Approaches for Skeletal Disorders

Room: 263/267
Chair: Steven C. Ghivizzani, PhD
Speakers: Christopher Evans, PhD - Tissue Regeneration Without Manufactured Scaffolds or Cell Culture
Dan Gazit, DMD, PhD - Genetically Engineered Mesenchymal Stem Cells as a Platform for Skeletal Tissue Engineering
Paul D. Robbins, PhD - Gene Therapy for Bone and Cartilage Regeneration
Edward M. Schwarz, PhD - Revitalization of Structural Allografts via Immobilized rAAV Gene Therapy

BREAK – 10:00 AM - 10:15 AM

SCIENTIFIC SYMPOSIA – 10:15 AM - 12:15 PM

Scientific Symposium 310
Embryonic Stem Cells and Tissue Engineering

Room: 230 Complex
Chair: Carolyn Lutzko, PhD
Speakers: Linda G. Griffith, PhD - Microscale Models of Liver for Analysis of Gene Therapy
Mervin Yoder, MD - Using RNA Interference Approaches to Modulate Gene Expression in ES Cells
Kyunghee Choi, PhD - Stepwise Commitment from Embryonic Stem to Hematopoietic and Endothelial Cells
Michael R. Linden, PhD - AAV-mediated Site-Specific Gene Addition in Embryonic Stem Cells

Scientific Symposium 311
Immunology of Gene Therapy: Immune Response to Gene Transfer Vehicles

Room: 240 Complex
Chair: Hildegund CJ Ertl, MD
Speakers: James M. Wilson, MD, PhD - T Cell Responses to Adenoviruses and AAV
Katherine A. High, MD - Immune Responses to Vector and Transgene Product in AAV-mediated Gene Transfer
Thierry C. Vandendriessche, PhD - Transduction of Antigen-Presenting Cells using Viral Vectors: A Double Edged Sword

Scientific Symposium 312
Neural Disorders: Inherited Metabolic Brain Disorders

Room: Ferrara Theater
Chair: Beverly L. Davidson, PhD
Speakers: Luigi Naldini, MD, PhD - HSC-based Gene Therapy of Leukodystrophies
John H. Wolfe, VMD, PhD - Gene Therapy for the CNS in Lysosomal Storage Disease
Paola Leone, PhD - Safety and Efficacy of AAV-mediated Gene Transfer for Canavan Disease
Ronald G. Crystal, MD - AAV-mediated Gene Transfer for Batten Disease

Scientific Symposium 313
Nonviral: Nonviral Gene Delivery: Towards Clinical Applications

Room: 260/264
Chair: Dexi Liu, PhD
Speakers: Lluis M. Mir, DSc - Electric Pulses-based Gene Transfer: Safety and Efficacy of Combinations of High Voltage and Low Voltage Pulses
Hans J. Lipps, PhD - A Novel Non-viral Episomal Vector as Expression System for Gene Therapy
Michael A. Barry, PhD - Endonucleases as Barriers to Gene Delivery
William M. Pardridge, MD - Intravenous, Non-Viral Gene Therapy of Brain in Rodents and Primates
SCIENTIFIC PROGRAM

FRIDAY, JUNE 3, 2005

Scientific Symposium 314
Respiratory Tract: Respiratory Epithelium as a Model for Mucosal Gene Delivery
Room: 263/267
Co-Chairs: Daniel Weiss, MD, PhD
Patrick L. Sinn, PhD
Speakers: Stephen Hyde, PhD - Non-viral Vectors for Airway Epithelium: The Pros and Cons
Larry G. Johnson, MD - Cell Biological Considerations in Gene Transfer to Respiratory Epithelia
Jim Hu, PhD - Viral Vectors for Airway Gene Delivery: Pros and Cons
Adriana Heguy, PhD - Microarray Analysis of Gene Expression in the Human Small and Large Airways to Identify Targets for Lung Disease

MEET-THE-INVESTIGATOR LUNCH SESSIONS – 12:30 PM - 1:30 PM

Meet-the-Investigator 320
Room: Washington Avenue A
Chair: Kenneth Cornetta, MD - Integrating Vectors: From Production to Clinic

Meet-the-Investigator 321
Room: Washington Avenue B
Chair: Cynthia Dunbar, MD - Insertional Mutagenesis and Retroviral Vectors

Meet-the-Investigator 322
Room: Washington Avenue C
Chair: Adrian J. Thrasher, MD, PhD - Clinical Trials for Immunodeficiency

SIMULTANEOUS ORAL ABSTRACT SESSIONS – 2:00 PM – 4:30 PM

Oral Abstract Session 330
RNA Virus Vectors: Integration and Vector Safety
Room: Ferrara Theater
Co-Chairs: Isabelle Riviere, PhD
Tal Kafri, PhD
(Abstracts #386 - #395)

Oral Abstract Session 331
AAV Vectors: Vector Development
Room: 240 Complex
Co-Chairs: Saswati Chatterjee, PhD
Guangping Gao, PhD
(Abstracts #396 - #405)

Oral Abstract Session 332
Adenovirus Vectors: Innate Immunity
Room: 120/124
Co-Chairs: Joseph T. Bruder, PhD
Andrea Amalfitano, DO, PhD
(Abstracts #406 - #415)

Oral Abstract Session 333
Inborn Errors of Metabolism - Liver and Storage Disorders
Room: 260/264
Co-Chairs: Seng H. Cheng, PhD
Brendan Lee, MD, PhD
(Abstracts #416 - #425)

Oral Abstract Session 334
Neurologic Disorders
Room: 265/266
Chair: Pedro Lowenstein, MD, PhD
(Abstracts #426 - #435)

Oral Abstract Session 335
Infectious Diseases and Vaccines: RNAi in Gene Therapy for Infections
Room: 230/231
Chair: Bruce E. Torbett, PhD, MSPH
(Abstracts #436 - #443)

Oral Abstract Session 336
Targeting of Adeno Viral Vectors and Cancer Treatment
Room: 123/127
Co-Chairs: Estuardo Aguilar-Cordova, PhD
William F. Benedict, MD
(Abstracts #444 - #453)

Oral Abstract Session 337
Advances in Hematologic Gene Therapy
Room: 263/267
Co-Chairs: Karen E. Pollok, PhD
Christof von Kalle, MD
(Abstracts #454 - #463)

Oral Abstract Session 338
Gene Regulation: Regulated Systems and Cell Engineering
Room: 261/262
Co-Chairs: David Spencer, PhD
James M. Wilson, MD, PhD
(Abstracts #464 - #473)
SCIENTIFIC PROGRAM

FRIDAY, JUNE 3, 2005

POSTER SESSION II – 4:00 PM - 7:30 PM
Hall 2

RNA Virus Vectors: Gene Transfer, Vector Production
(Abstracts #474 through #496)

AAV Vectors: Vector Biology
(Abstracts #497 through #523)

Adenovirus Vectors: Applications
(Abstracts #524 through #555)

Naked DNA Gene Transfer: Optimizing Expression, Animal Models
(Abstracts #556 through #575)

Lipid Mediated Gene Transfer: Non-Viral Delivery System
(Abstracts #576 through #596)

Inborn Errors of Metabolism: Hemophilia
(Abstracts #597 through #615)

Cardiovascular: Angiogenesis and Vascular Gene Therapy
(Abstracts #616 through #639; and Abstract #921)

Neurologic: Pain
(Abstracts #640 through #646)

Neurologic: Spinal Cord Injury and Motor Neuron Disease
(Abstracts #647 through #654)

Neurologic: Advances in Vectors, Delivery and Imaging
(Abstracts #655 through #662)

Neurologic: Sensory
(Abstracts #663 through #671)

Gene Therapy for Connective Tissue
(Abstracts #672 through #690)

Cancer - Immunotherapy: Cytokine Transgenes and Vaccinations
(Abstracts #691 through #709)

Cancer - Targeted Gene Therapy: Targeting of Non-Adenoviral Vectors
(Abstracts #710 through #732)

Gene Regulation: Overcoming Obstacles to Gene Transfer and Expression
(Abstracts #733 through #754)

EVENING SESSION – 8:30 PM – 10:00 PM

Evening Session 340
Immune Responses in Neurologic Gene Therapy - Basic Bench Perspectives
Target Audience: Anyone interested in immune responses as well as neural gene therapy in the brain.
Room: 261/262
Co-Chairs: Pedro Lowenstein, MD, PhD
Hildegund CJ Ertl, MD
Speakers: Ronald G. Crystal, MD
Paola Leone, PhD
James Wilson, MD, PhD
Michael Kaplitt, MD, PhD
John Iacomini, PhD
Roland W. Herzog, PhD
Katherine A. High, MD
Samuel D. Rabkin, PhD
Jean Bennett, MD, PhD
David J. Fink, MD
Ronald Mandel, PhD
SCIENTIFIC PROGRAM

SATURDAY, JUNE 4, 2005

SCIENTIFIC SYMPOSIA – 8:00 AM - 10:00 AM

Scientific Symposium 400
Cancer: Clinical Trials
Room: Ferrara Theater
Chair: Steven M. Albelda, MD
Speakers: Daniel H. Sterman, MD - Gene Therapy Clinical Trials for Pleural Malignancies
John J. Nemunaitis, MD - Oncolytic Viral Gene Delivery Vehicles
Chiara Bonini, MD - Suicide Gene Therapy in Stem Cell Transplantation
Kristen Hege, MD - GVAX Cancer Vaccines - Clinical Development

Scientific Symposium 401
Cardiovascular: Advances in Cardiovascular Cell and Gene Therapy
Room: 230 Complex
Co-Chairs: Lawrence C. Chan, MD, Douglas Losordo, MD
Speakers: Lawrence C. Chan, MD - Novel Therapy of Obesity: Implications for Cardiovascular Disease
Douglas Losordo, MD - Adult Stem Cells: Fact or Fiction
H. Kirk Hammond, MD - Adenylyl Cyclase Gene Transfer for Heart Failure

Scientific Symposium 402
Hemopoietic: Advances in Stem Cell Self Renewal
Room: 260/264
Co-Chairs: David W. Emery, PhD, Karen E. Pollok, PhD
Speakers: R. Keith Humphries, MD, PhD - Emerging Strategies for High Level Expansion of Hematopoietic Stem Cells
C. Anthony Blau, MD - Pharmacologically Regulated Cell Therapy
Akihiro Kume, MD, PhD - On-demand Expansion of Genetically Corrected Blood Cells

Scientific Symposium 403
Oligonucleotide Based Therapies: Emerging Applications of Oligonucleotide Therapeutics
Room: 240 Complex
Chair: Dieter Gruenert, PhD
Speakers: Bruce Sullenger, PhD - Aptamer-Antidote Pairs as a Novel Approach to Safer Drug Design
Mark A. Kay, MD, PhD - RNAi Based Approaches for Treatment of Human Hepatitis Virus Infection
Peter Glazer, MD, PhD - Targeted Gene Correction via Triplex Formation
Andrew Ellington, PhD - Aptamer Inhibition of HIV

Scientific Symposium 404
Viral: Vector Cell Interactions
Room: 263/267
Chair: Kenneth Cornetta, PhD
Speakers: Glen R. Nemerow, PhD - Adenovirus Cell Entry Mechanisms
Arun Srivastava, PhD - AAV-mediated Transgene Expression: Second-strand Synthesis Versus Strand-annealing
Nicholas Muzyczka, PhD - Targeting AAV Vectors

BREAK – 10:00 AM - 10:15 AM

SIMULTANEOUS ORAL ABSTRACT SESSIONS – 10:15 AM - 12:15 PM

Oral Abstract Session 410
DNA Viruses: Vaccines, HSV, Baculovirus and SV40
Room: 240 Complex
Co-Chairs: David Krisky, MD, PhD, Yoshinaga Sacki, MD, PhD
(Abstracts #755 - #762)

Oral Abstract Session 411
Naked DNA Gene Transfer: Optimizing Expression/Animal Models
Room: 260/264
Co-Chairs: Ronald K. Scheule, PhD, Carol H. Miao, PhD
(Abstracts #763 - #770)

Oral Abstract Session 412
Lipid Mediated Gene Transfer: Non Viral Delivery Systems
Room: 263/267
Co-Chairs: Ralph W. Paul, PhD, James E. Hagstrom, PhD
(Abstracts #771 - #778)
AMERICAN SOCIETY OF GENE THERAPY • Final Program

SCIENTIFIC PROGRAM

SATURDAY, JUNE 4, 2005

Oral Abstract Session 413
Molecular Conjugates: Therapeutic Applications
**Room:** 120/124
Co-Chairs: Lonnie D. Shea, PhD
Theresa M. Reineke, PhD
(Abstracts #779 - #786)

Oral Abstract Session 414
Muscle and Connective Tissue: Gene Therapy for Muscular Dystrophy
**Room:** 230/231
Co-Chairs: Olivier Danos, PhD
Paula Clemens, MD
(Abstracts #787 - #794)

Oral Abstract Session 415
Cellular Immunotherapy of Cancer Using Genetically Modified Effectors
**Room:** Ferrara Theater
Chair: Michael Jensen, MD
(Abstracts #795 - #802)

Oral Abstract Session 416
Cancer-Apoptosis and Suicide
**Room:** 123/127
Co-Chairs: Sunil Chada, PhD
Patricia Yotnda, PhD
(Abstracts #803 - #810)

Oral Abstract Session 417
Gene Therapy Approaches to Pulmonary Disease
**Room:** 261/262
Co-Chairs: Terence R. Flotte, MD
Christine L. Halbert, PhD
(Abstracts #811 - #818)

MEET-THE-INVESTIGATOR LUNCH
SESSIONS – 12:30 PM - 1:30 PM

Meet-the-Investigator 420
**Room:** Washington Avenue A
Chair: Harry L. Malech, MD - Ex Vivo Hematopoietic Stem Cell Therapy for Inherited Immune Disease

Meet-the-Investigator 421
**Room:** Washington Avenue B
Chair: R. Scott McIvor, PhD - Drug Resistance and Transposons in Gene Therapy Research

Meet-the-Investigator 422
**Room:** Washington Avenue C
Chair: Luigi Naldini, MD, PhD - Lentiviral Vectors for Stem Cell Gene Transfer
SCIENTIFIC PROGRAM

SATURDAY, JUNE 4, 2005

POSTER SESSION III – 4:00 PM - 7:00 PM
Hall 2

RNA Virus Vectors: Safety, Therapeutics
(Abstracts #823 through #846; Abstract #822 has been moved to Poster Session I)

AAV Vectors: Vector Development
(Abstracts #847 through #871)

Adenovirus Vectors: Vector Technologies
(Abstracts #872 through #893)

Inborn Errors of Metabolism: Liver and Pancreatic Diseases
(Abstracts #894 through #915)

Cardiovascular: Myocardial Gene Therapy
(Abstracts #916 through #938; Abstract #921 has been moved to Poster Session II)

Neurodegenerative Diseases
(Abstracts #939 through #960)

Neurologic: Other
(Abstracts #961 through #972)

Gene Therapy for Infections and Vaccines
(Abstracts #973 through #995)

Infectious Diseases and Vaccines: RNAi for Gene Therapy of Infections
(Abstracts #996 through #1003)

Cancer - Targeted Gene Therapy: Targeting of Adenoviral Vectors
(Abstracts #1004 through #1029; and Abstract #328)

Hematologic - New Advances
(Abstracts #1030 through #1049)

Gene Regulation: Regulated Systems and Tissue Specific Expression
(Abstracts #1050 through #1073)

SUNDAY, JUNE 5, 2005

SCIENTIFIC SYMPOSIUM – 8:30 AM - 10:00 AM

Scientific Symposium 500
Young Investigators
Room: America's Ballroom
Co-Chairs: Joseph C. Glorioso, PhD
David Bodine, PhD
Speakers:
Chiara Bonini, MD - Gene Transfer into Peripheral Blood T Lymphocytes: Clinical Benefits and Safety Profile
Introduced by Fabio Candotti, MD
Michael Kaplitt, MD, PhD - Development of Human Gene Therapy for Neurodegenerative Disorders
Introduced by Ronald G. Crystal, MD

SIMULTANEOUS ORAL ABSTRACT SESSIONS – 10:15 AM - 12:15 PM

Oral Abstract Session 510
Clinical Gene Therapy
Room: 240 Complex
Co-Chairs: Arthur Nienhuis, MD
Terence R. Flotte, MD
(Abstracts #1074 - #1079)

Oral Abstract Session 511
AAV Vectors: Disease Applications
Room: 260/264
Co-Chairs: Roland W. Herzog, PhD
Xiao Xiao, PhD
(Abstracts #1080 - #1087)

Oral Abstract Session 512
Adenovirus Vectors: Adaptive Immunity and Vaccines
Room: 120/124
Co-Chairs: Victor Krasnykh, PhD
Robin J. Parks, PhD
(Abstracts #1088 - #1095)

Oral Abstract Session 513
Naked DNA Gene Transfer: Chromosomal Integration
Room: 263/267
Co-Chairs: R. Scott McIvor, PhD
Cary Harding, MD
(Abstracts #1096 - #1103)

Oral Abstract Session 514
Molecular Conjugates: Vector Engineering
Room: 265/266
Co-Chairs: Suzie H. Pun, PhD
David V. Schaffer, PhD
(Abstracts #1104 - #1111)
SCIENTIFIC PROGRAM

SUNDAY, JUNE 5, 2005

Oral Abstract Session 515
Cardiovascular: Angiogenesis and Vascular Gene Therapy
Room: 261/262
Chair: Andrew H. Baker, PhD
(Abstracts #1112 - #1119)

Oral Abstract Session 516
Muscle and Connective Tissue: Gene Therapy for Connective Tissue
Room: 230/231
Co-Chairs: Paul D. Robbins, PhD
Steven C. Ghivizzani, PhD
(Abstracts #1120 - #1127)

Oral Abstract 517
Cancer - Immunotherapy: Genetic Strategies to Induce and Augment Anti-Tumor Immunity
Room: 123/127
Chair: Michael Jensen, MD
(Abstracts #1128 - 1135)

12:15 PM – ASGT 8TH ANNUAL MEETING CONCLUDES

See you in 2006!
ASGT 9th Annual Meeting
May 31 – June 4, 2006
Baltimore, Maryland
AMERICA’S CENTER FLOOR PLAN

LEVEL 1

America’s Center Floor Plan
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HOUSING INFORMATION

A RENAISSANCE GRAND HOTEL*
   Headquarters Hotel
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   St. Louis, Missouri 63101
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C HOLIDAY INN SELECT DOWNTOWN
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   St Louis, Missouri 63101
   Phone: 314-421-4000
MAP OF ST. LOUIS
ABSTRACTS

THURSDAY, JUNE 2

Oral Abstract Session 240
RNA Virus Vectors: Gene Transfer and Gene Expression

Room: 260/264

1  2:00 PM
Persistent Gene Expression in Mouse Nasal Epithelia Following Baculovirus GP64 Pseudotyped FIV-Based Gene Transfer
Patrick L. Sinn, Erin R. Burnight, Melissa A. Hickey, Gary W. Blissard, Paul B. McCray, Jr.

2  2:15 PM
Cellular Mechanisms Involved with Lentiviral Gene Transfer
Shangming Zhang, Karen Pollok, Lakshmi Sastry, Lionel Berthoux, Jeremy Luban, Kenneth Cornetta

3  2:30 PM
Effects of the eHS4 Insulator Elements on Transgene Expression and Biological Titer of Lentiviral Vector
Hideki Hanawa, Takashi Shimada

4  2:45 PM
Optimization of Globin Lentiviral Vector Design for the Treatment of β-Thalassemia
Leszek Lisowski, Stefano Rivella, Michel Sadalain

5  3:00 PM
Differential Expression of PIT1 and PIT2 after G-CSF Mobilization Is Associated with Efficient Gene Transfer Using GALV-Pseudotyped gammaretroviral Vectors
Brian C. Beard, Pau Mezquita, Julia C. Morris, Hans-Peter Kiem

6  3:15 PM
In Vivo Adult Stem Cell Gene Transfer in Mice by In Situ Delivery of a Self-Inactivating Lentiviral Vector Using Intrafemoral Injection
D. Nicole Worsham, Kimberly Bohn, David Kuhel, David A. Williams, Christof von Kalle, Dao Pan

7  3:30 PM
Transduction of Quiescent Human Cells Using an SIVsmmPbj-Derived Lentiviral Vector
Matthias Schweizer, Nina Wolfrum, Michael Muehlebach, Julia Kaiser, Silke Schuele, Klaus Cichutek

8  3:45 PM
Potent Inhibition of HIV-1 Replication by Lentiviral Vectors Carrying Anti-Vif siRNA
S. Li, G. Wang, K. Meinking, M. J. Li, J. Ji, J. A. Zaia, J. J. Rossi

9  4:00 PM
Coronaviruses: Development of Novel Oncolytic Vectors
Tom Würdinger, Hélène M. Verheije, Victor W. van Beusechem, Xander A. M. de Haan, Winold R. Gerritsen, Peter J. M. Rottier

10 4:15 PM
The Effect of Globin Locus Control Region (LCR) Elements or the MSCV-LTR on Promoter Trapping by Integrated Lentiviral Vector Genomes
Byoung Y. Ryu, Derek A. Persons, Arthur W. Nienhuis

Oral Abstract Session 241
AAV Vectors: Vector Biology

Room: 240 Complex

11  2:00 PM
An Additional Role of Cellular FKBP52 in Recombinant Adeno-Associated Virus 2 Vector-Mediated Gene Transfer and Transgene Expression
Weihong Zhao, Li Zhong, Jianqing Wu, Liyuan Chen, Keyun Qing, Kirsten A. Weigek-Kelley, Steven H. Larsen, Arun Srivastava

12  2:15 PM
Self-Complementary AAV2 Vectors Transduce Liver with the Same Efficiency as AAV8: The Critical Role of Second-Strand Synthesis in AAV Biology
Zhijian Wu, Hongzhe Duan, R. Jude Samulski

13  2:30 PM
Persistence of AAV Capsid in Transduced Cells
Mo-Ying Hsieh, Fayaz R. Khazi, Alexander Schlachterman, Yi-Lin Liu, Katherine A. High

14  2:45 PM
rAAV2 Integration Junctions Isolated without In Vivo Selection Bias Show a Preference to 5’ Regions of Open Reading Frames
Fayaz R. Khazi, Alexander Schlachterman, Katherine A. High

15  3:00 PM
Large-Scale Analysis of Adeno-Associated Virus Vector Integration Sites in Normal Human Cells
Daniel G. Miller, Grant D. Trobridge, Lisa M. Petek, Michael A. Jacobs, Rajinder Kaul, David W. Russell

16  3:15 PM
Single-Polarity Recombinant Adeno-Associated Virus 2 Vector-Mediated Transgene Expression In Vitro and In Vivo: Mechanism of Transduction
Li Zhong, Xiaohuai Zhou, Yanjun Li, Keyun Qing, Richard J. Samulski, Arun Srivastava

17  3:30 PM
Visualization of the Intranuclear rAAV Replication Centers and Their Co-Localization with Double-Stranded DNA Break Repair Proteins
Cervelli Tiziana, Lorena Zentilin, Alessandro Marcell, Mauro Giacci
ABSTRACTS

THURSDAY, JUNE 2

18  3:45 PM
Facilitation of AAV Genome Circularization by Host Cell Factors Involved in DNA Replication and Double-Strand Break Repair
Vivian W. Choi, Douglas M. McCarty, Richard J. Samulski

19  4:00 PM
rAAV-2 Transduction of Primitive Human Hematopoietic Stem Cells Capable of Serial Engraftment in Immune Deficient Mice
Helicia Paz, Leah Santat, Lijing Li, Christie Wong, Stephen J. Forman, K. K. Wong, Suswati Chatterjee

20  4:15 PM
Targeted Gene Delivery to Embryonic Stem Cells
Els Henckaerts, Nadja Zeltner, Steven Kattman, Nathalie Dutheil, Peter Ward, Marion Kennedy, Nathalie Clement, Patricia Rebollo, Gordon Kelley, R. Michael Linden

Oral Abstract Session 242
Adenovirus Vectors: Vector Technology
Room: Ferrara Theater

21  2:00 PM
Application of Splice Acceptor Transposons to the Development of Oncolytic Viruses
Fang Jin, Peter Kretschmer, Cecile Chartier, Irene Kuhn, Terry Hermiston

22  2:15 PM
Generation of Circular Sleeping Beauty DNA Transposition Substrates by RU486-Induced Flp Recombination In Vitro – Steps towards a Single Integrating Adeno-Transposon Vector
Jacob G. Mikkelsen, Stephen R. Yant, Anja Ehrhardt, Mark A. Kay

23  2:30 PM
Frequency of Random and Targeted Chromosomal Integration of Helper-Dependent Adenoviral Vector
Fumi Ohbayashi, Eini Aizawa, Atsuhiro Kishimoto, Ko Mitani

24  2:45 PM
Context-Specific Peptide-Presenting Phage Libraries for Adenoviral Vector Targeting
Debadyuti Ghosh, Michael A. Barry

25  3:00 PM
Genetic Incorporation of HSV-1 Thymidine Kinase into the Adenovirus Protein IX for Functional Display on the Virion
Jing Li, Long Le, Don A. Sibley, J. Michael Mathis, David T. Curiel

26  3:15 PM
Microsphere Delivery of Adenoviral-Liposome Complexes Reduces Adenoviral Immunogenicity and Allows Successful Re-Administration of the Virus without Loss of Gene Expression Efficiency
Jason C. Steel, John C. Morris, Mark A. Burton, Heather M. A. Cavanagh, Wouter H. J. Kalle

27  3:30 PM
A Novel “Geneti-Chemical” Platform for Flexible and Efficient De- and Retargeting of Adenovirus Vector Particles
Florian Kreppel, Judith Gackowski, Erika Schmidt, Stefan Kochanek

28  3:45 PM
Adenovirus Targeting Via Leucine Zipper Peptide-Mediated Ligand Attachment
Joel N. Glasgow, Nikolay Korokhov, David T. Curiel

29  4:00 PM
Redirection of Adenoviral Tropism through Capsomers Other Than Fiber Is Generally Inefficient and Dependent on Ligand-Receptor Biology
Samuel K. Campos, Michael A. Barry

30  4:15 PM
AdenoLibrary for Construction and Selection of Targeted Adenoviruses
Sam C. Noureddini, Alexander Krendelshchikov, Vera Simonenko, Joanne T. Douglas, David T. Curiel, Nikolay Korokhov

Oral Abstract Session 243
Inborn Errors of Metabolism - Stem Cells and Blood Disorders
Room: 120/124

31  2:00 PM
Somatic Gene Therapy for ADA-SCID Following Cessation of PEG-ADA and Use of a Mild Conditioning Regime
Hubert B. Gaspar, Emma Bjorkegren, Katherine Parsley, Kimberly C. Gilmour, Jo Sinclair, Fang Zhang, Lynette D. Fairbanks, Doug King, Graham Davies, Paul A. Vey

32  2:15 PM
Tolerance after Neonatal Gene Transfer of a Human Factor IX-Expressing Retroviral Vector May Involve Clonal Deletion in C3H Mice and Is Effective in Hemophilia B Dogs
Lingfei Xu, Mark E. Haskins, Timothy C. Nichols, Manxue Mei, Putty O’Donnell, Dwight A. Belling, Stephanie McCorquodale, Katherine P. Ponder
ABSTRACTS

THURSDAY, JUNE 2

33 2:30 PM
Non-Random Genomic Distribution of Retrovirus Vector Integration in Successful SCID-X1 Gene Therapy
Kerstin Schwarzwaelder, Manfred Schmidt, Steven Howe, Hanno Glimm, Claudia Prinz, Manuela Wisser, Annette Deichmann, Sonja Schmidt, Bobby Gaspar, Adrian Thrasher, Christof von Kalle

34 2:45 PM
Transposon-Based Gene Therapy of Hemophilia A Targeting Endothelial Cells in Neonates
Li Liu, Bradley S. Fletcher

35 3:00 PM
Generalized Detoxification Associated with Engraftment of Gene-Corrected Repopulating Cells Achieved in ADA-SCID Patients by Stem Cell Gene Therapy without Myelopreparative Pre-Conditioning
Makoto Otsu, Satoru Nakaijima, Miyuki Kida, Yoshhiro Maeyama, Nariaki Toda, Norikazu Hatan, Nobufuki Kawamura, Motohiko Okano, Ryoji Kobayashi, Osamu Tatsuzawa Masafumi Onoda, Fabio Cantor, Michael S. Hershfield, Yukio Sakiyama, Tadashi Ariga

36 3:15 PM
Improved Lentiviral Vectors for Systemic Gene Transfer in the Absence of an Immune Response
Brian D. Brown, Ehud Hauben, Angelo Lombardo, Lucia Sergi Sergi, Maria Grazia Roncarolo, Luigi Naldini

37 3:30 PM
A Novel Form of Enzyme Replacement Therapy for ADA-Deficiency: In Vivo Transduction by Neonatal Injection of Lentivirus Expressing ADA
Denise A. Carbonaro, Xiangyang Jin, Denise Petersen, Donald B. Kohn

38 3:45 PM
Inhibition of Apolipoprotein B-100 Expression by Lentivirus Mediated RNA Interference in Mice after Stem Cell Transduction and Transplantation
Mikko P. Turunen, Petri I. Makinen, Pia Leppanen, Juulia Einback, Suvi Koota, Teru Vatanen, Seppo Yla-Herttuala

39 4:00 PM
Effects of Long-Term Expression of Activated Murine FVII in Normal and Hemophilic Mice
Majed N. Aljamali, Paris Margaritis, Rodney M. Camire, Katherine A. High

40 4:15 PM
Enhanced Factor IX Delivery from Bioengineered Hybrid Human Skeletal Muscle Co-Expressing VEGF
Lieve Thorrez, Herman Vandenburgh, Desire Collen, Janet Shansky, Thierry VandenDriessche, Marinee Chuah

Oral Abstract Session 244
Cardiac Gene Transfer
Room: 123/127

41 2:00 PM
High Efficiency, Catheter-Based, Gene Transfer to the Large Animal Heart
David M. Kaye, Kenneth Chien, Masahiko Hoshijima, Krisztina Zsebo, John Power

42 2:15 PM
Uniform Scale-Independent Gene Transfer to Striated Muscle after Transvenular Extravasation of Vector

43 2:30 PM
Targeting the Biology of Heart Disease: Engineered Zinc Finger Protein Repressors of Phospholamban as a Potential Therapy for Congestive Heart Failure

44 2:45 PM
Development of AAV-Mediated Gene Therapy for Murine Models of Genetic Diseases Affecting the Heart
Christina A. Pacak, Cathryn Mah, Gabriel Gaidosh, Melissa Lewis, Raquel Torres, Kevin Campbell, Glenn A. Walter, Barry J. Byrne

45 3:00 PM
Adenoviral Gene Transfer of SERCA2a Restores Mechanical and Energetic Left Ventricular Function in Spontaneously Diabetic Rats
Susumu Sakata, Yuri Sakata, Elie R. Chenah, Prabhu M. Padmanabhan, Djamal Lebeche, Miyoko Takaki, Federica del Monte, Roger J. Hajjar

46 3:15 PM
In Vivo Adenoviral Gene Transfer of Activated PI3-Kinase Rescues Cardiac Dysfunction and Injury Induced by Ischemia-Reperfusion after Chronic Cardiac Akt Activation
Tomohisa Nagoshi, Takashi Matsu, Takuma Ayaama, Ling Li, David A. Kass, Hunter C. Champion, Anthony Rosenzweig

47 3:30 PM
Ex Vivo Hypothermic Recirculatory Adenoviral Gene Transfer to the Transplanted Pig Heart
ABSTRACTS

THURSDAY, JUNE 2

48 3:45 PM
The Long-Term Effects of SERCA2a Overexpression in Large Animal Model of Heart Failure
Yoshiaki Kawase, Ryuichi Yoneyama, Kozo Hoshino, Jennifer McGregor, Thanh-Thao Ton-Nu, Tom G. Neilan, Robert A. Levine, Judy Hung, Motoya Hayase, Federica del Monte Roger Hajjar

49 4:00 PM
Evaluation of Catheter-Based Deliveries of 10um Gelatin Hydrogel Microsphere, a Novel Non-Viral Vehicle, in a Porcine Heart
Kozo Hoshino, Ryuichi Yoneyama, Yoshiaki Kawase, Alec M. De Grand, John V. Frangioni, Yasuhiko Tabata, Takeshi Kimura, Toru Kita, Roger J. Hajjar, Motoya Hayase

50 4:15 PM
Transcript Profiling Identifies Novel Role for SGK1 in Promoting Cardiomyocyte Survival
Takuma Aoyama, Takashi Matsui, Mikhail Novikov, Anthony Rosenzweig

Oral Abstract Session 245
Neurodegenerative Diseases
Room: 263/267

51 2:00 PM
Adenoviral BDNF/Noggin-Induced Neurogenesis from Endogenous Neural Stem Cells Delays Motor Impairment and Extends Survival in a Transgenic Model of Huntington’s Disease
Sung-Rae Cho, Eva Chmielnicki, Aris Economides, Steven A. Goldman

52 2:15 PM
AAV-Delivered RNAi Improves Cellular and Motor Phenotypes in a Mouse Model for Huntington’s Disease
Scott Q. Harper, Patrick D. Staber, Xiaohua He, Ines H. Martins, Qinwen Mao, Henry L. Paulson, Robert M. Kotin, Beverly L. Davidson

53 2:30 PM
Post-Transcriptional Suppression of Striatal Mutant Huntingtin Leads to Mild Phenotypic Improvements in the R6/1 Mouse
Edgardo Rodriguez, Eileen Donovan-Wright, Kevin Nash, Alfred S. Lewin, Ronald J. Mandel

54 2:45 PM
Lentiviral-Mediated Silencing of SOD1 through RNA Interference Delays Disease Onset and Progression in a Mouse Model of ALS
Cedric Raoul, Patrick Aebischer

55 3:00 PM
Lentiviral-Mediated Silencing of Mutant SOD-1 Using RNAi Causes Long Term Correction of ALS in a Transgenic Mouse Model
Gareth S. Ralph, Pippa A. Radcliffe, Denise M. Day, Janine M. Carthy, Susan M. Kingsman, Kyrkios A. Mitropoulos, Mimoun Azzouz, Nicholas D. Mazarakis

56 3:15 PM
Amelioration of Neurodegenerative and Neuropathological Alterations in a Transgenic Model of Alzheimer’s Disease: Targeting BACE1 with Small Interfering RNAs
Oded Singer, Robert A. Marr, Edward Rockenstein, Leslie Crews, Fred H. Gage, Inder M. Verma, Eliezer Masliah

57 3:30 PM
Therapy for Alzheimer’s Disease by Neprilysin Gene Transfer
Robert A. Marr, Edward Rockenstein, Atish Mukherjee, Mark S. Kindy, Louis B. Hersh, Fred H. Gage, Eliezer Masliah, Inder M. Verma

58 3:45 PM
Rett Syndrome Is Reversible and Treatable by MeCP2 Gene Therapy into the Striatum in Mice
Ken-ichiro Kosai, Akira Kusagai, Takeo Isagai, Koji Hirata, Satoshi Nagano, Yoshiyu Murofushi, Tomoyuki Takahashi, Sachio Takashima, Toyojiro Matsuishi

59 4:00 PM
rAAV-Mediated Nigral Parkin Over-Expression Is Neuroprotective in the 6-OHDA Rat Model of Parkinson’s Disease
Fredric P. Manfredsson, Alfred S. Lewin, Nicholas Muzyczka, Corinna Burger, Ronald J. Mandel

60 4:15 PM
Recovery of Vestibular Function Induced by math1 Gene Delivery
Hinrich Staecker, Mark Praetorius, Douglas E. Brough

Oral Abstract Session 246
Gene Therapy for Infections and Vaccines
Room: 230/231

61 2:00 PM
Higher In Vivo Maintenance, Biodistribution and Immunopotency of Dendritic Cell Vaccines Self-Differentiated through Lentiviral Vector Programming
Richard C. Koya, Takahiro Kimura, Antoni Ribas, Nori Kasahara, Renata Stripecke

62 2:15 PM
Transduction of CD34+ Hematopoietic Progenitor Cells with a SIV-Based Lentiviral Vector Expressing Antisense SIV Env Protects CD4+ T Cell Progeny from SIVmac239 Infection
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63  2:30 PM
A Nonhuman Primate Model for Testing Immuno Gene Therapies for AIDS Using Gene-Protected T Cells
Joern E. Schmitz, Felix Hermann, Sebastian Neuwurz, Melissa Ricketts, Patricia Schult-Dietrich, Alexander Szyroki, Robert P. Johnson, Dorothee von Laer

64  2:45 PM
Characterization of the Mechanism of Action of the Anti-HIV-1 Transgene F12-Vif
Rosella Lupe, Giuliana Vallanti, Maurizio Federico, Fulvio Marvillo, Chiara Bovelenta

65  3:00 PM
Protective Immunity Against Anthrax Lethal Toxin in Mice Immunized with an Ad-Based Vaccine Vector Expressing Lethal Factor
Julie L. Boyer, Tiffany B. Niven, Neil R. Hackett, Ronald G. Crystal

66  3:15 PM
A Protective Vaccine for the Rapid Response to Pandemic Avian Influenza
Wentao Gao, Adam Sooffee, Xiuhua Liu, Angela Montecalvo, Yuniko Matsuoka, Paul D. Robbins, Roben O. Donis, Jacqueline M. Katz, Simon Barratt-Boyce, Andrea Gambotto

67  3:30 PM
Protective Immunity Against Yersinia pestis in Mice Immunized with an Ad-Based Vaccine Vector Expressing the Y. pestis F1 and V Antigens
Maria Chiuchiolo, Julie L. Boyer, Tiffany Niven, Anja Krause, Neil R. Hackett, Ronald G. Crystal

68  3:45 PM
Development of a Novel Non-Viral DNA Vaccine-Delivering Gene into the Nucleus of Dendritic Cells
Takashi Nakamura, Kentaro Kogure, Rumiko Moriguchi, Shiroh Futaki, Masahiro Fujimuro, Hideyoshi Yokosawa, Tsukasa Seya, Hideyoshi Harashima

Oral Abstract Session 247
Cancer-Targeted Gene Therapy: Non Adenoviral Targeting of Vectors and Non Viral Vectors
Room: 265/266

69  2:00 PM
Tumor-Targeting Immune Gene Therapy by Mesenchymal Stem Cells Expressing CX3CL1
Hong Xin, Masahiko Kanehira, Hiroyuki Mizuguchi, Takao Hayakawa, Yoshihiro Nukiwa, Yasuo Saijo

70  2:15 PM
Systemic Administration of siRNA Against EWS-FLI1 Using a Targeted, Non-Viral Formulation Inhibits Growth in a Disseminated Murine Model of Ewing’s Sarcoma
Jeremy D. Heidel, Siwen Hu-Lieskovan, Derek W. Bartlett, Timothy J. Triche, Mark E. Davis

71  2:30 PM
Intratumoral Delivery of Small Interfering RNA for Inhibition of Tumor Progression in Mice
Yuki Takahashi, Makiya Nishikawa, Yoishinobu Takakura

72  2:45 PM
Capsid Modifications Overcome Low Heterogeneous Expression of Heparan Sulfate Proteoglycan That Limits AAV2-Mediated Gene Transfer and Therapeutic Efficacy in Human Ovarian Carcinoma
Jeffrey S. Bartlett, Wenfang Shi

73  3:00 PM
Fully Retargeted Oncolytic Measles Viruses for Cancer Therapy
Takahumi Nakamura, Mary Harvey, Suzanne Greiner, Kah-Wyhe Peng, Stephen J. Russell

74  3:15 PM
Specific Delivery of hTRAIL and Inhibition of Ewing’s Sarcoma Growth by Transplantation of Viral Vector-Transduced Bone Marrow-Derived Mesenchymal Stem Cells (MSC)
Jeffrey S. Bartlett, Wenfang Shi, Carmel Lawrenza

75  3:30 PM
Molecular Imaging of an EGFP-DRD2-Expressing Vaccinia Virus
Navneet Mehta, Nan Tang, Hui Deng, Deborah Scollard, James Jonkman, Brian C. Wilson, Raymond M. Reilly, Judith A. McCarr

76  3:45 PM
New Strategy To Improve Imaging and Retargeting of HSV-1 Vectors
Paola Grandi, Trevor D. McKee, Wilson Mok, Juliet L. Fernandez, Xandra O. Breakefield, Rakesh K. Jain

77  4:00 PM
Efficient Transduction and Therapy of Malignant Glioma by Lentiviral Vectors Pseudotyped with LCMV Glycoproteins
Hrvoje Miletic, Yvonne Fischer, Harald Neumann, Tavan Giroglou, Manuel M. Hermann, Werner Stenzel, Maria A. Rueger, Yannick Waczogers, Uwe Himmelreich, Mathias Hoehn Andreas H. Jacobs, Martina Deckert, Dorothee von Laer
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78 4:15 PM
Expression of the Herpes Simplex Virus Thymidine Kinase Gene by a Promoter Region of the Human SPARC Gene Inhibits Human Melanoma Cell Growth In Vitro and In Vivo
Maria V. Lopez, Patricia V. Blanco, Diego L. Viale, Eduardo A. Cafferata, David Gould, Yuti Chernajovsky, Osvaldo Podhajcer
Oral Abstract Session 248
Gene Regulation: Keeping Genes On and Turning Them Off
Room: 261/262

79 2:00 PM
Real Time Fluorescent Tracking of Dynamic Variegation by Retrovirus Vectors in Stem Cells
James Ellis, John Ramunas, Liam Kelly, Tanya Sukonnuk, Eric Jervis

80 2:15 PM
Insulation of Oncoretrovirus Vectors with the cHS4 Chromatin Insulator Is Associated with an Open Chromatin Histone Code
Changlong Li, David W. Emery

81 2:30 PM
A Retrovirus Vector Screen for the Identification of Novel Chromatin Insulators and Enhancers
Amy C. Groth, George Stamatoyannopoulos, David W. Emery

82 2:45 PM
Targeted Pancreatic Cancer-Specific Gene Therapy Controlled by a Molecular-Engineered Human Cholecystokinin Type-A Receptor (CCKAR) Promoter in In Vivo Imaging Models
Xiaoming Xie, Zheng Li, Qingqing Ding, Hus-Ping Kuo, James L. Abbruzzese, Mien-Chie Hung

83 3:00 PM
Persistent and Position Independent Transgene Expression in Erythroid Cells Transduced by Lentiviral Vectors
Annarita Miccio, Francesco Lotti, Claudia Rossi, Giuliana Ferrari

84 3:15 PM
Assessing Hemostatic Efficacy of Continuous Expression of Factor VIIa after AAV-Mediated, Liver-Directed Gene Transfer in Hemophilia A and B Mice
Alexander Schlachterman, Paris Margaritis, Majed Aljamali, Jianhua Liu, Valder Arndt, Katherine High

85 3:30 PM
Development of Zinc Finger Nucleases for Therapeutic Gene Correction of Sickle Cell Anemia

86 3:45 PM
Transcriptional Gene Silencing in Human Cells
Zhaoxia Chen, Ali Elsani, Rigs Art, Rossi J. John, Kevin V. Morris

87 4:00 PM
Determinants of Toxicity Induced by In Vivo Expression of Short Hairpin RNA from AAV Vectors
Dirk Grimm, Konrad L. Streetz, Kusum Pandey, Theresa A. Storm, Mark A. Kay

88 4:15 PM
Driving shRNA Transcription from PolII Promoters in Mammalian Cells
Jeffery C. Giering, Mark A. Kay

Poster Session I
Exhibit Hall 2
RNA Virus Vectors: Gene Expression

89
Amelioration of Gamma Retroviral Silencing during Embryonic Stem Cell-Derived Hematopoiesis: Preclinical Assessment of Safety-Improved Vectors
Ali Ramezani, Teresa S. Hawley, Robert G. Hawley

90
In Vitro and In Vivo Gene Expression of Lentiviral Vectors in CD4+ T Cells
Kit L. Shaw, Karen Pepper, Denise Petersen, Vesa Kaartinen, Donald B. Kohn

91
Insertion of the cHS4-Insulator into Lentiviral Vectors: Use of Real-Time PCR in Order To Analyze the Effects on Transgene Expression and Viral Titre
Johan Jakobsson, Troels Tøstrup Nielsen, Nina Rosenqvist, Cecilia Lundberg

92
Efficient Transgene Expression in Central Nervous System through a Non-Integrative Lentiviral Vector
Stephanie Philippe, Che Seguerer, Sebastien Bonnel, Christelle Véto, Martine Barkats, Caroline Petit, Marc Abitbol, Chansy Sarkis, Jacques Mallet

93
Diminished Mobilization of Self-Inactivating (SIN) Lentiviral Vectors Containing Globin Regulatory Elements Compared to Those Containing a Retroviral Long Terminal Repeat
Hideki Hanawa, Derek A. Persons, Takashi Shimada, Arthur W. Nienhuis

94
Transgene Expression with Integration-Deficient HIV-1 Based Lentiviral Vectors Expressing Class I Integrase Mutants
G. Joseph, J. Marsh, T. Johnson, K. Cornetta
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95 Transduction and Expression of shRNAs Via Lentiviral Vectors Bearing a U6 RNA Polymerase III Promoter, but Not an H1 Promoter, Induce Elevated Apoptosis in Human Primary Peripheral Blood T Lymphocytes
Dong Sung An, Xiao-Feng Qin, Si-Hua Mao, Vincent C. Auyeung, David Baltimore, Irvin S. Y. Chen

96 Ocular Gene Transfer Using EIAV Vectors and an RPE-Specific Promoter
Shu Kachi, Katie Binley, Naoyasu Umeda, Hideo Akiyama, Raquel Lima e Silva, Wei-Hong Xiao, Michiko Kachi, Margaret Esapa, Sharifah Iqball, Stuart Naylor Peter A. Campochiaro

97 Type I Interferon Response Against Virus Vectors and Non-Viral Gene Transfer
Riikka Pellinen, Olli Meriläinen, Saara Lehmusvuara, Tiula Salonen, Katja Hakkinen, Tanja Hakkarainen, Elisa Vähäkangas, Kari Airenne, Ilkka Julkunen, Ari Hinkkanen Jarno Wählörs

98 Stable Lentivirus-Mediated RNA Interference: Comparison of U6 and H1 Promoters in Endothelial Cells and GFP Knockdown in Mouse Brain
Petri I. Makinen, Jonna K. Koponen, Anna-Mari Timonen, Tarja M. Malm, Jari Koistinaho, Mikko P. Timonen, Seppo Yla-Herttuala

99 Use of a Liver-Specific Lentivirus Vector for Characterization of Hepatocyte-Like Cells from Human Differentiated Embryonic Stem Cells (hESC)
Yuyou Duan, Naoki Yamamoto, Jian Wu, Sanjeev Gupta, Mark A. Zern

100 Development of an Integrase Deficient FIV Vector for Transient Gene Expression
Marisa Banasik, Melissa Hickey, Paul McCray Jr.

101 Development of Lentiviral Vectors with Respiratory Epithelial Specific Transgene Expression
Dinithi Senadheera, Benjamin Hendrickson, Denise Petersen, Karen Pepper, Carolyn Lutzko

102 Overexpression of the Myostatin Antagonist Follistatin in Normal Myoblasts Genetically Modified with a Lentivirus
Basma F. Benabdallah, Joel Rousseau, Manaf Bouchentouf, Jacques P. Tremblay

103 PGK and CMV Promoters Exert the Strongest Activity in Lentiviral Gene Transduction of Myeloid Cells Including Mature Neutrophils
Yasushi Soda, Kenzaburo Tani, Xiaojin Li, Yuansong Bai, Seok-Goo Cho, Muneosu Furutani, Minghan Chen, Seichiro Kobayashi, Hiroyuki Miyoshi, Hideki Sumimoto Shoichi Ohga, Toshiro Hara, Arinobu Tojo, Shigetaka Asano

104 Stable High Level Gene Expression by Minimum Sized Murine Leukemia Virus (MLV)-Based Retroviral Vectors in the Mouse Model
Seong Shin Yu, Youngtae Hong, Sujeong Kim, Chang Wan Joo, Nam Kyung Yoon, Sunyoung Kim

105 Comparative Study on the Gene Expression Profile of HIV-1 Based Lentiviral Vectors Transduced Human and Murine Cells
Yuan Zhao, Selina Azam, Robin Thorpe

106 Measles Virus Host Defense Evasion Proteins V and C Have Also Transcription Modulation and Infectivity Factor Functions
Patricia Devaux, Warrangkha Songsungthong, Christoph Springfeld, Veronika von Mesling, Roberto Cattaneo

107 Sustained Stable Gene Expression Induced by a Cytoplasmic RNA-Virus Vector Based on Mutant Sendai Virus Strain cl.151
Ken Nishimura, Hiroaki Segawa, Takahiro Goto, Mariko Morishita, Takanasu Sakauchi, Tetsuya Yoshida, Kozo Takayama, Mahito Nakashima

108 The Complete Nucleotide Sequence of Sendai Virus Isolate BB1 and Comparison with Other Isolates
Yu Yang, Lufeng Ren, Xiaoyan Dong, Xiaoheng Wu

109 Expression of Porcine a(1-3)galactosyltransferase in Non-Human Primate Hematopoietic Progenitors Following Retroviral Mediated Gene Transfer
Lorenzo Benatulis, John Iacomini

(Note: Abstract 822 has been moved from Poster Session III to Poster Session I and will follow Abstract 109. See page 96 for abstract title and authors.)
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AAV Vectors: Disease Applications

110 Performance of Different AAV Serotype Vectors Following Injection into the Deep Cerebellar Nuclei of ASMKO Mouse Brain
James C. Dodge, Jennifer Clarke, Anthony Song, Jie Bu, Qi Zhao, Tatyana V. Takis, Denise Griffis, Lamya S. Shihabuddin, Catherine R. O’Riordan, Marco A. Passini Ed H. Schuchman, Gregory R. Stewart

111 AAV7, 8, and 9 Are More Efficient and Less Immunogenic Vectors for Muscle-Directed Gene Therapy for Hemophilia B
Lili Wang, Jean-Pierre Louboutin, Yan Li, James M. Wilson

112 Strong Transgene-Specific Immune Responses Can Be Elicited with Novel Adeno-Associated Virus Vectors in Mice
Yan Zhi, Julie Johnston, Di Wu, Joanna Figueredo, Martin Lock, Peter Bell, Guangping Gao, James M. Wilson

113 Manipulating AAV2 Tropism for Enhanced Delivery of AAV Vectors to the Spinal Cord
Adam Davis, Matthew Stachler, Wenfang Shi, James Liu, Nicholas Boulis, Jeffrey Bartlett

114 Efficient Arterial Formation at the Sites of Adult Neo-Angiogenesis Requires the Recruitment of Bone Marrow Cells through the Neuropilin-1 (NP-1) Receptor
Serena Zacchigna, Ennio Tasciotti, Nikola Arsic, Oreste Sorace, Marini Cecilia, Silvia Pardini, Debora Petroni, Claudia Kusmic, Lucia Pattarini, Silvia Moimas GianMario Sambuceti, Mauro Giacca

115 In Vivo Expression of Human Cob(I)alamin Adenosyltransferase Using rAAV Serotypes 1, 2 and 8, an Approach to Gene Therapy for Methylmalonic Aciduria (MMA)
Kirsten E. Erger, Thomas J. Conlon, Travis L. Cossette, Thomas A. Bobik, Terence R. Flotte

116 Functional Comparison of Four CFTR rAAV Vectors with Alternative Transcriptional Elements in Cell Lines and Polarized Human CF Airway Epithelia
Liang Zhang, Benjamin Dutzar, Keith Munson, Barrie Carter, John F. Engelhardt

117 AAV-Mediated Erythropoietin Gene Transfer Protects from Genetic and Light-Induced Retinal Degeneration
Maria Carmela Allocca, Tonia S. Rex, Luciano Domenici, Jean Bennet, Victor M. Rivera, Alberto Auricchio

118 Self-Complementary AAV Vectors Pseudotyped with Either Serotype 5 or 8 Capsids Give Therapeutic Human Factor IX Levels at Significantly Reduced Doses in Non-Human Primates
John T. Gray, Andrew M. Davidoff, Amit C. Nathwani

119 Intraperitoneal Transduction of Adeno-Associated Virus 2 Expressing Angiostatin and Endostatin Synergistically Augments Paclitaxel Therapy and Tumor-Free Survival in a Mouse Model of Epithelial Ovarian Cancer
Tatyana Isayeva, Changshun Ren, Selvarangan Ponnazhagan

120 Stimulation of the Synthesis of Type-II Collagen and Extracellular Matrix in Human Normal and Osteoarthritic Articular Cartilage Explants In Situ by Overexpression of Human sox9 by rAAV-Mediated Gene Transfer
Henning Madry, Tanja Thurn, Dieter Kohn, Ernest F. Terveilliger, Magali Cucchiarini

121 VEGF-Induced Angiogenesis Does Not Have a Favorable Effect on Muscle Perfusion: A Relevant Role of Long-Term Angiopoietin-1 Expression in the Formation of Functional Blood Vessels
Serena Zacchigna, Ennio Tasciotti, Nikola Arsic, Oreste Sorace, Marini Cecilia, Silvia Pardini, Debora Petroni, Claudia Kusmic, Lucia Pattarini, Silvia Moimas GianMario Sambuceti, Mauro Giacca

122 Enhanced Expression of Glutamate Decarboxylase 65 Significantly Improves Rat Parkinsonian Symptom
Boyoung Lee, Young Nam, Sung Jin Kim, Jin Hwan Oh, Yoon Hee Choi, Jin Woo Chang, Heuiran Lee

123 rAAV-pdx-1 Augmented the Insulin-Producing Cells
Hua Li, Karen S. Lam, Sidney Tam, Weidong Xiao, Paul Tam, Ruian Xu

124 The “Perivascular Pump” Driven by Arterial Pulsation Is a Powerful Mechanism for the Distribution of Therapeutic Molecules within the Brain
Piotr Hadaczek, Hanna Mirek, Laszlo Tamas, Martha C. Bolon, Charles Noble, John W. Park, Krystof Bankiewicz

125 AAV Vector-Mediated Neonatal Gene Transfer: Efficient Transgene Expression in Muscles after Intraperitoneal Cavity Vector Injection
Tsuyoshi Ogura, Hiroaki Mizukami, Jun Mimuro, Takashi Okada, Hiromi Hamada, Akihiro Kume, Hiroaki Yoshikawa, Yoichi Sakata, Keiya Ozawa
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126 The Formation of Stable and Mature Blood Vessels Requires the Proper Timing of VEGF Expression
Sabrina Tafuro, Serena Zacchigna, Lorena Zentilin, Marina Dupas, Mauro Giacca

127 Intraarticular Gene Transfer of Rat TNFR:Fc Suppressed CIA Arthritis
Ai Zhi Zhao, Kai Gao, Xiao Yan Dong, Jian Qiang Peng, Xiao Bing Wu

128 Transduction of Hepatic Oval Cells, Stem Cells, In Vitro by Adeno-Associated Virus Vectors
Hong You, Ping Wang, Jidong Jia, Liu Yong, Paul L. Hermonat

129 Characterization of a Murine Adeno-Associated Virus (AAV-mo.1) Capsid
Michael Lochrie, Gwen Tatsuno, Alejandra Arbetman, Peter Smith, Jennifer Wellman, Shang-zhen Zhou, Glenn Pierce, Peter Colosi

130 Development of Vectors Utilizing Strong Neuronal Cell Type Specific Promoters for High Level Expression of Beta Glucuronidase in the Central Nervous System
Tupur Husain, John H. Wolfe

131 Recombinant Adeno-Associated Virus 2-Mediated Transfer of the Human Superoxide-Dismutase Gene Does Not Confer Radiosensitivity on HeLa Cervical Carcinoma Cells
Marlon R. Veldwijk, Carsten Herskind, Stephanie Laufs, W. Jens Zeller, Stefan Fruhaufl, Federik Wenz

132 An Optimized CsCl Gradient -Based Method to Generate High Concentration AAV Vector Stocks of High Purity and In Vivo Potency
Mario Cooper, Bernd Hauck, Jeff Watkins, Megan Erne, Federico Mingozzi, Roland Herzog, Fraser Wright

133 Comparison of Adeno-Associated Viral Vector Serotypes 1, 2, 5 and 6 for Gene Transfer to Neurons and Glia In Vitro
Doug B. Howard, Juan Li, Yun Wang, Brandon K. Harvey

134 Toxicology and Biodistribution Studies of a Recombinant Adeno-Associated Virus 1 (rAAV1) Alpha-1 Antitrypsin (AAT) Vector
Amy E. Poirier, Thomas J. Conlon, Lynn A. Combee, Kirsten E. Erger, Terence R. Flotte

135 AAV-Mediated Gene Delivery To Evaluate the Biology of an Inherited Macular Degeneration
Nicholas W. Keiser, Daniel C. Chung, Waiying Tang, Zhanrong Wei, Albert Maguire, Jean Bennett, Jeannette L. Bennicelli

136 Recombinant Adeno-Associated Virus 2-Mediated Ectopic Expression of a4b1 Integrin in Mouse Mesenchymal Stem Cells Enhances Repopulation to Bone upon Syngeneic Transplantation in an Immunocompetent Mouse Model
Sanjay Kumar, Selvarangan Ponnazhagan

Adenovirus Vectors: General Biology and Host Response

137 Localization of Regions in CD46 That Interact with Adenovirus
Anuj Gaggar, Dmitry M. Shayakhmetov, M. Kathryn Liszewski, John P. Atkinson, Andre Lieber

138 Blood Factors Affect Adenovirus Infectivity and Biodistribution In Vivo
Dmitry M. Shayakhmetov

139 Priming and Boosting Efficiency of Chimp and Human Adenoviral Vectors in the Setting of Pre-Existing Immunity in Non-Human Primates
Joanita Figueredo, Roberto Caleco, Jim Miller, Gary Kobinger, Rebecca Grant, Guangping Gao, James M. Wilson

140 Primary Adenovirus Specific Cytotoxic T Lymphocyte Response Occurs after Viral Clearance and Liver Enzyme Elevation
Jian Chen, Allan J. Zajac, Sylvia A. McPherson, Xin Xu, PingAr Yang, Qi Wu, David T. Curiel, John D. Mountz

141 Adenovirus Vaccine to Ebola Virus: Mechanism of Protection and Impact of Pre-Existing Immunity to the Vaccine Carrier

142 Molecular and Macromolecular Alterations of Recombinant Adenoviral Vectors Do Not Eliminate Changes in Hepatic Drug Metabolism
Shellie M. Callahan, Michael P. Boquet, Piyanuch Wonganan, Maria A. Croyle
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143 Regulated, High Capacity Adenoviral Vectors Mediated Long-Term Gene Expression in the Brain Even in the Presence of a Peripheral Immune Response to Adenovirus
Weidong Xiong, Shyam Gowdethnana, James F. Curtin, Carlos Barcia-Gonzalez, Jeffrey M. Zinger, Gwendalyn D. King, Sandra A. Sciascia, Mariana Puntel, Maricela Candolfi, Donna Palmer Philip Ng, Pedro R. Lowenstein, Maria G. Castro

144 In Vivo Studies on the Toxicity of Adenovirus Vectors for Kupffer Cells
Elanchezhiyan Manickam, Jeffrey S. Smith, Jie Tian, Andrew P. Byrnes

145 Evaluation of Biodistribution and Safety of Adenovirus Vectors Containing B-Group Fibers after Intravenous Injection into Baboons
Shaoheng Ni, Kathrin Bernt, Zong-Yi Li, Hans-Peter Kiem, Andre Lieber

146 T Cell Response Against the Cystic Fibrosis Transmembrane Conductance Regulator (CFTR) Gene
Maria Limberis, Joanita Figueredo, James M. Wilson

147 Adenovirus Vector E4 Gene Regulates Connexin 40 and 43 Expression in Endothelial Cells Via PKA and PI3K Signal Pathways
Joseph Cheng, Fan Zhang, George Lam, David Jin, Loic Vincent, Neil R. Hackett, Shiyang Wang, Lauren M. Young, Barbara Hempstead, Ronald G. Crystal Shahin Rafii

148 The Transgene Cassette Is Not Fully Responsible for Alterations of Renal Cytochrome P450 Expression after Systemic Administration of Recombinant Adenovirus
Hong T. Le, Michael P. Boquet, Erin A. Clark, Shellie M. Callahan, Maria A. Croyle

149 Transient Kupffer Cell Saturation, Via Adenovirus or Adenoviral Protein, Enhances Expression of Reporter Virus
Viraj P. Mane, Christian Clarke, Lali Medina-Kame, Milton Finegold, Brendan Lee

150 Pre-Existing Nab and Neutralization of Therapeutic Dose Adenovirus Serotypes In Vitro
Edward C. Nwanegbo, Wentao Gao, Andrea Gambotto, Paul Robbins

151 Evaluation of a Serotype 35 Fiber Containing Adenovirus Vector for Vaccination
Nelson C. Di Paolo, Shaoheng Ni, Anuj Gaggar, Zong-Yi Li, Andre Lieber

152 Coxsackievirus-Adenovirus Receptor (CAR) and αβ, or αβ, Integrin Independent Internalization of Porcine Adenoviral Vectors: Implications in Gene Therapy
Dinesh S. Bangari, Suresh K. Mittal

153 Adenoviral Gene Therapy Vectors Expressing the Adenovirus Death Protein Demonstrate Increased Transgene Expression In Vivo as Demonstrated by Dose-Volume Histogram Analysis of Reporter Gene Activity
Kenneth N. Barton, Stephen L. Brown, Jae Ho Kim, Hans Stricker, Kastytis Karsvelis, Mei Lu, Svend O. Freytag

154 Relationship between the Expression of Membrane Receptors (CAR, Integrin αvβ3 and Integrin αvβ5 ) and Adenoviral Transduction Efficiency
Hiroyuki Inoue, Terumasa Hisano, Yikoh Nakazaki, Gaku Sakaguchi, Ryo Kurita, Koichi Takayama, Yoichi Nakaniishi, Kenzaburo Tani

“Other” DNA Virus Vectors

155 Oral Delivery of Recombinant Vaccinia Viruses Expressing Adjuvanted Islet Autoantigens Protects NOD Mice from Autoimmune Diabetes
William H. R. Langridge, Bela Denes, Nadia Fodor, Valentina Krausova, Tatiana Timiryasova, David Henderson, John Hough, Jie Yu, Istvan Fodor

156 Update on Genomic HSV Vector-Related Cytotoxicity: A Vector Preserving the Differentiation Potential of Embryonic and Adult Adipose Stem Cells
Julie Fradette, Steven K. Wendell, Jim Wechuck, David M. Krisly, Darren Wolfe, William F. Goins, Joseph C. Glorioso

157 Quantitation of Long-Term Gene Expression from the HSV-1 Latency-Associated Promoter in the Mouse Brain Following Intracranial Injection
Bradford K. Berges, Ananya Gupta, John H. Wolfe, Nigel W. Fraser

158 Gene Therapy for Chronic Pain Control: Perspectives of HSV-1 Mediated Gene Transfer to Supraspinal Centres
Isabel Martins, Marta Pinto, Steven Wilson, Deolinda Lima, Isaura Tavares
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159 Unexpected Genetic Alterations Occur in Untargeted Genes during the Construction of Viral Vectors
Megan J. Dambach, Jordan Trecki, Natalia Martin, Nancy S. Markovitz

160 Systemic Oncolytic Herpes Simplex Virus Therapy of Spontaneously Arising Prostate Cancer in Transgenic TRAMP Mice
Susan Varghese, Samuel D. Rabkin, Petur G. Nielsen, Renbin Liu, Wenzheng Wang, Robert L. Martuza

161 Targeting HSV Infection to gD Receptor-Negative Cells Using Soluble gD Receptors
Heechung Kwon, Qing Bai, Kelly Felment, Hyun-Jung Baek, Gyuyoung Kim, William F. Goins, Justus B. Cohen, Joseph C. Glorioso

162 Targeted HSV-1 Infection by Adapters for Glycoprotein D: Variable Infection Efficiency Dependent on Ligand Choice
Kenji Nakano, William F. Goins, Justus B. Cohen, Joseph C. Glorioso

163 Engineered of a Highly Efficient Cell Cycle Regulatable-HSV-1 Amplicon Viral Vector
Grace Ying Wang, Ioy A. W. Ho, Kam M. Hui, Paula Y. P. Lam

164 Bioluminescence Imaging in the CNS after HSV Amplicon Vector Delivery
Sam Wang, Lisa Pike, Jeremy Petravicz, Xandra O. Breakefield

165 Tracking of Baculovirus Biodistribution In Vivo by Magnetic Resonance Imaging
Jani K. Raty, Thomas Wirth, Kari J. Airenne, Toulia Huhtala, Timo Liimatainen, Ale Narvanen, Juhana Hakumaki, Seppo Yla-Herttuala

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295
Adeno-Associated Virus-Mediated Transfer of the Genes Encoding Cryptic Kringle Fragments of Apolipoprotein (A) Suppresses Tumor Growth and Metastasis In Vivo
Kyuhyun Lee, Sunn-Tae Yun, Eui-Cheol Jo

296
Effective Suppression of Breast Cancer by Downregulation of VEGF Signaling
Yi Lu, Jun Zhang, Derrick J. Beech, Lisa K. Jennings

297
Radiovirotherapy in Ovarian Cancer Using Recombinant Measles Expressing Sodium Iodide Symporter
Kosei Hasegawa, Hooi-Tin Ong, David Dingli, Takaumi Nakamura, Stephen J. Russell, Kah Whye Peng

298
Gene Therapy Against Prostate Cancer Using the HSV- tk/Prodrug System Followed by Prostatectomy
Augusto Rojas-Martinez, Laura K. Aguilar, Rocio Ortiz-Lopez, Jacinto Esteban-Maria, Juan F. Gonzalez-Guerrer0, Raquel Garza-Guijardo, Juan P. Flores-Gutierrez, E. Brian Butler, Hugo A. Barrera-Saldana, Estuardo Aguilar-Cordova

299
Increasing Gene Transfer and Oncolytic Potency of Adenoviruses in Orthotopic Models of Gastric Cancer
Loita Kangasniemi, Anna Kaneru, Mari Raki, Tiuli Ranki, Merja Sarkioja, Tiula Kiviluoto, Ulf-Hakan Stenman, Henrik Alfihan, Hongju Wu, David T. Curiel Akseli Hemminki

300
Adenoviral Delivery of the Gene Encoding Secretable Trimeric TRAIL Induces Apoptosis and Suppresses Human Malignant Glioma In Vivo
Moonsup Jeong, In-Ho Kwon, Chae-Young Kim, Dai-Wu Seol, Paul D. Robbins, Byoung-Moon Kim
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THURSDAY, JUNE 2

301
Down-Regulation of bcl-xl Gene Expression with bcl-xl siRNA Vectors In Vitro and In Vivo
Xiaobo X. Cao, Philip Rascoe, Jonathan Daniel, Charles Rodarte, W. Roy Smythe

302
Herpes Simplex Virus Thymidine Kinase/Ganciclovir Therapy Mediated by Transferrin-Associated Lipoplexes in a Murine Model for Oral Cancer
Silvia S. Neves, Sergio Simões, Maria C. Pedroso de Lima

303
Validating CD46 as a Possible Mechanism Contributing to MV-Edm Oncolytic Specificity
Hooi-Tin Ong, Stephen J. Russell, Kah-Whye Peng

304
Gene Therapy of Malignant Brain Tumor by a Novel Double-Stranded Adeno-Associated Viral (AAV) Vector Carrying the Decorin Gene
Chi-Hsien Wang, Jun-Ming Han, Kai-Yun Chen, Shi-Yuan Cheng, Xiao Xiao, Hsin-I Ma

305
A Safe TRAIL to Lung Tumor Specificity
Juan Shi, Dexian Zheng, Yanxin Liu, Mai Har Sham, Paul Tam, Farzin Farzaneh, Ruian Xu

306
A Novel Thymidine Kinase in Enhanced Gap Junction Mediated Suicide Gene Therapy
Tomas J. Ekstrom, Zahidul Khan, Zoran Gojkovic, Mette Willer, Per M. Almqvist

307
The Tumor-Selective Viral Protein Apoptin Is a Potential Gene Therapeutic Agent for Prostate Cancer Therapy
Xiang Liu, Ahmed El-Zawhry, David Holman, Saeed Elojeiny, Alicja Bielawaska, Jack Bielawaska, Mahvash Tavassoli, James S. Norris

308
In Vivo Enhancement of HSV-TK/GCV Cancer Gene Therapy with Polyamine Biosynthesis Inhibition
Tiina Wahlors, Anne Karppinen, Juhani Jänne, Leena Alhonen, Jarmo Wahlors

309
STAT5 Phosphorylation in Malignant Melanoma Is Mediated through SRC and JAK-1 Kinases and Exerts Anti-Apoptotic Effects
Alireza Mirmohammadsadegh, Walter Bardenheuer, Mohamad Hassan, Annett Guetha, Dennis Selimovic, Roya Doroudi, Nicola Schmittner, Sandeep Nambiar, Alessandra Marini, Thomas Ruzicka Ulrich R. Hengge

310
Cancer - Targeted Gene Therapy: Targeting of Non-Viral Vectors

311
Discovered Peptide Mediates Specific Binding to Primary Medullary Thyroid Carcinoma after Systemic Injection
Miriam Boekmann, Gero Hilken, Aaron N. Cranston, Matthias Drosten, Bruce A. J. Ponder, Anke Schmidt, Brigitte M. Putzer

312
Oncolytic Adenovirus CG5757 Preferentially Infects Primary Human Tumor Tissues and Shows Strong Anti-Tumor Activity in Tumor Models Following Systemic Administration
Yuanhao Li, Trini Arroyo, Stephen Thorne, Tony Reid, Natalie Nguyen, Neeraja Idamakanti, Melinda VanRoey, Gail Colbern, De-Chao Yu

313
Human Plasminogen Kringle 5-Engineered Murine Mammary Cancer Cells Arrest Tumor Growth and Promote Long-Term Survival
Sabrina R. Perri, Moira François, Jacques Galipeau

314
Efficacy of Group I Intron-Based Trans-Splicing Ribozyme in Tumor-Targeted Gene Therapy
Byung-Su Kwon, Ju Hyun Kim, Jin-Sook Jeong, In-Hoon Kim, Seong-Wook Lee

315
The Epithelial Glycoprotein-2 Promoter for Highly Specific and Efficient Cancer Gene Therapy for a Broad Range of Tumors
Willemijn M. Gommans, Gerben Duns, Pamela M. J. McLaughlin, Simone J. van Eert, Masato Yamamoto, David T. Curiel, Hidde J. Haisma, Marianne G. Rots

316
Highly Specific Expression of the Luciferase Gene in Lungs of Naïve Nude Mice Directed by Prostate Specific Antigen Promoter
Hongwei Li, Jin Zhong Li, Gregory A. Helm, Dongfeng Pan

317
Dendritic Cell-Based Genetic Immunotherapy for Prostate Cancer
Briana J. Williams, Nikolay Korokhov, Susan Boling, Linda Li, Michael Mathis, David T. Curiel

318
Raf-1 siRNA Inhibits Tumor Growth In Vivo
Qixin Leng, A. James Mixson

319
Tumor Growth Inhibition from Tumor Targeted Delivery of Diphtheria Toxin Gene
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319
ColoAd1, a Chimeric Ad11p/Ad3 Oncolytic Virus for the Treatment of Colon Cancer
Irene Kuhn, Paul Harden, Maxine Bauzon, Terry Hermiston

320
Antitumor Effect of Intrahepatic Interleukin-12 Expression from a Regulable Plasmid Vector in Murine Models of Primary and Metastatic Liver Cancer
Maider Zahala, Ruben Hernandez-Alcoceba, Margarita Ezay, Ignacio Melero, Christine Perret, Ivan Penuelas, Jesus Prieto, Gabriela M. Kramer

321
Splice-Switching of the Prostate Specific Membrane Antigen (PSMA) in LnCap Prostate Cancer Cells
Tiffany L. Williams, Ryszard Kole

322
Tumor Specific Activation of Irinotecan by a Carboxylesterase Fused with Anti CEA scFv
Junji Uchino, Koichi Takayama, Tomomichi Sone, Akiko Harada, Taishi Harada, David T. Curiel, Yoichi Nakaniishi

323
Transcriptional Targeting of Viral Gene Transfer by Human Hexokinase II Promoter
Ann-Marie Maatta, Sanna Korja, Tanja Hakkarainen, Jarmo Wahlors, Kimmo Mäkinen, Esko Alhava, Riikka Pellinen

324
TRAIL Gene Therapy Is a Promising Therapy for Bladder Cancer
Ahmed M. El-Zawahry, Christina Voelkel-Johnson

325
Identification of Genes Expressed Specifically in Esophageal Cancer Cells
Fumiaki Sati, John M. Abraham, Jing Yin, Takatsugu Kan, Yuriko Mori, Stephen J. Meltzer

326
Gene Therapeutic Overexpression of MDR1 Affects Transcriptional Regulations of Proteins Involved in Apoptosis and Detoxification
Patrick Maier, Li Li, Katharina Fleckenstein, Stephanie Laufs, Stefan Freuauf, Jens Zeller, Frederik Wenz, Carsten Herskind

327
The Effect of NO on HIF-Mediated Gene Expression: Chemical Sources of NO Versus Genetic Regulation of iNOS
Rana S. Al-Assah, Rachel L. Cowen, Edwin C. Ching, Ian J. Stratford

(Note: Abstract 328 has been moved from Poster Session I to Poster Session III and will follow Abstract 1029.)

328
An Orthotopic Murine Model of Advanced Breast Cancer for Imaging and Comparison of Targeting Moieties
Tiuli Ranki, Anna Kanerva, Karl von Smitten, Merja Sarkioja, Lotta Kangasniemi, Mari Raki, Pirjo Laakkonen, Hongtu Wu, David T. Curiel, Steven Goodison Akseli Hemminki

329
Use of Recombinant Non-Pathogenic Bacteria as Vectors for Hypoxia Targeted Gene Expression for Cancer Gene Therapy
Asferd Mengesha, Ludwig Dobois, Brad G. Wouters, Phillippe Lambin, Jan Theys

Hematologic - Disease Models

330
Lentiviral Vector Mediated Hematopoietic Stem Cell Gene Therapy Combined with Non-Lethal Conditioning Restores T Cell Function in the Murine Model of Wiskott-Aldrich Syndrome
Francesco Marangoni, Loïc Dupré, Samantha Saramuzzza, Sara Trifari, Raisa Jofra Hernández, Adrián Thrasher, Anne Galy, Alessandro Aiuti, Luigi Naldini, Maria Grazia Roncarolo

331
Lentivirus-Mediated Ex Vivo Gene Therapy in ADA-Deficient SCID Mice
Alessandra Mortellaro, Raisa Jofra Hernández, Matteo Guerrini, Antonella Tabucchi, Filippo Carlucci, Antonia Fellenzi, Luigi Naldini, Claudio Bordignon, Maria Grazia Roncarolo, Alessandro Aiuti

332
Chronic Granulomatous Disease Gene Therapy Functionally Corrects the Phenotype of Polymorphonuclear Leukocytes (PMN)
Ulrich Siler, Marion G. Ott, Stefan Stein, Eike Kanaus, Maja Rutishauser, Corinne Wenk, Dieter Hoelzer, Manuel Grez, Reinhard Seger

333
Preferential Targeting of Transcriptional Start Sites after Retroviral-Mediated T-Cell Gene Therapy for Adenosine Deaminase Deficiency
Daniele Moratto, Jayashree G. Jagadeesh, Akihiro Konno, Marita Bosticardo, Gregory E. Crawford, Ingeborg Holt, Linda M. Mun, Shepherd H. Shurman, Tyra Wolfsberg, Fabio Candotti

334
Treatment of Anemia in Mice with Chronic Renal Failure Utilizing Erythropoietin-Secreting Genetically-Engineered Murine Bone Marrow Stromal Cells
Nicoletta Eliopoulos, Raymonde F. Gagnon, Moira Francois, Jacques Galipeau
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335  Correction of a Murine Model of Von Willebrand Disease by Gene Transfer
Robert G. Pergolizzi, Jin Guang, Diane Chan, Denisa Wagner, Peter Lenting, Ronald G. Crystal

336  Correction of Chronic Granulomatous Disease by Gene Therapy
Manuel Grez, Marion G. Ott, Stefan Stein, Ulrich Siler, Ulrike Koehl, Hana Kunkel, Andrea Schilz, Klaus Kuehlke, Dieter Hoelzer, Reinhard Seger

337  Towards Gene Correction Therapy for Wiskott-Aldrich Syndrome with Engineered Zinc Finger Nucleases
Lei Zhang, Fyodor D. Urnov, Jeffrey C. Miller, Christian Beausejour, Ya-Li Lee, Jeremy Rock, Kenneth Kim, Michael C. Holmes, Philip D. Gregory

338  Non-Viral Gene Transfer into Hematopoietic Cells of Artemis Deficient ScidA Mice Using the Sleeping Beauty Transposon System
Andy Wilber, Shannon Buckley, Uma Lakshmipathy, Joel Frandsen, Morton J. Cowan, R. Scott McIvor

339  Long-Term Protein Transduction Domain Mediated Intra-Cellular Delivery of Purine Nucleoside Phosphorylase (PNP) Corrects PNP Deficiency in Mice
Ana Toro, Eyal Grunebaum

340  Drug-Selectable as Well as Therapeutic Gene Transfer into Hematopoietic Stem Cells May Be Essential in Gene Therapy for Inherited Bleeding Disorders of Large Animals
David A. Wilcox, Juan Fang, Lily M. Du, Mary K. Boudreaux

341  Lentiviral Vector-Mediated Gene Therapy as Treatment for Wiskott-Aldrich Syndrome (WAS): Pre-Clinical Studies in Human Cell Lines and WASp +/- Mice

342  Human Erythropoietin Gene Therapy for Patients with Chronic Renal Failure

343  Repair of Thalassemic β0-Globin pre-mRNA by Antisense AAV and Lentiviral Vectors in Cell Culture
Thipparat Suwanmanee, Vivian W. Choi, Richard J. Samulski, Tal Kafri, Ryszard Kole

344  Application of a suicide Gene to X-SCID Gene Therapy
Tori Uchiyama, Satoru Kamaki, Masahuni Onodera, Du Wei, Looi Chung Yeng, Yoichi Sasahara, Sigeru Tsuchiya

345  Gene Therapy for Wiskott-Aldrich Syndrome Using Lentiviral Vectors: Evidence for Efficacy and Safety after Transduction of Human T Cells and Hematopoietic Stem Cells
Loï Dupré, Samantha Scaramuzzo, Francesco Marangoni, Sara Trifari, Silvana Martino, Shigeru Tsuchiya, Adrian Thrasher, Anne Galy, Luigi Naldini, Alessandro Aiuti Maria Grazia Roncarolo

346  Gene Correction of X-Linked SCID Using Engineered Zinc Finger Nucleases and Integration Defective Lentiviral Delivery
Angelo Lombardo, Christian Beausejour, Fyodor D. Urnov, Jeffrey C. Miller, Michael C. Holmes, Philip D. Gregory, Luigi Naldini

347  Long-Term Gene Expression and Phenotypic Correction of FANCC Deficient Lymphoblastoid Cells Using the Sleeping Beauty Transposon System
Shannon A. Wadman, Karl J. Clark, Perry B. Hackett, R. Scott McIvor, Jeffrey J. Essner

348  Integration Site Analysis of a Helper-Dependent Adenovirus Vector Containing AAV ITRs and 22 kb of the Human Globin LCR
Hongjie Wang, Tobias Leege, Dmitry Shayakhmetov, Qiliang Li, Thalia Papayannopoulou, George Stamatoyannopoulos, Andre Lieber

349  Partial Correction of IL-12 Receptor beta-1 (IL-12Rb1) Deficiency in Mice upon Transplantation of Retrovirally Transduced Hematopoietic Stem Cells
Marita Bosticardo, Charles Scanga, Francesco Novelli, Jean-Laurent Casanova, Fabio Candotti

350  Gene Therapy Approaches to Pulmonary Disease
Uta Griesenbach, on behalf of the UK Cystic Fibrosis Gene Therapy Consortium
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THURSDAY, JUNE 2

351 Repeat Administration of Polyethylenimine (PEI) Aerosols of Plasmid DNA to the Murine Lung Is Associated with a Loss of Gene Transfer Efficiency
Lee A. Davies, Stephen C. Hyde, Deborah R. Gill

352 Gene Transfer of Angiogenic Inhibitors Suppresses Lung Tumor Growth in Spontaneous Pulmonary Metastasis Mouse Model
Kexia Cai, Mai Har Sham, Weidong Xiao, Paul Tam, Ruian Xu

353 Retrovirally-Marked Human Bone Marrow Derived Mesenchymal Stem Cells Attenuate Radiation Induced Pneumonitis in a Xenotransplant Model
Andrew Hope, Todd E. Meyerrose, Jan A. Nolta

354 Correction of Respiratory Function by Recombinant AAV1 Mediated Gene Therapy in a Murine Model of Glycogen Storage Disease Type II
Cathryn Mah, Kerry O. Cresawn, Lara R. DeRuisseau, David Fuller, Barry J. Byrne

355 Towards Cystic Fibrosis and Airway Gene Therapy: Evaluation of EGFP Gene Expression in Murine Nasal Airways Mediated by Simian Immunodeficiency Virus Vectors Pseudotyped with Sendai Virus Glycoproteins F and HN
Katsuyuki Mitomo, Makoto Inoue, Yasuji Ueda, Mie Ikegami, Uta Griesenbach, Eric W. F. W. Alton, Mamoru Hasegawa

356 Sonoporation Increases Non-Viral Gene Transfer to the Murine Lung
Stefania Xenariou, Haidong Liang, Uta Griesenbach, Ray Farley, Luci Somerton, Charanjit Singh, Po-Wah So, Amy Herihir, Stefano Ferrari, Duncan Geddes Martin Blomley, Eric Alton

357 IL-17 Induces Migration of Adult Marrow Stromal Cells to Lung Epithelium
Roberto Loi, Jennifer Jaskolka, Robert Prenovitz, Ying Jian You, Steven Brody, Daniel J. Weiss

358 Gene Transfer to the Airway Epithelium Mediated by a Third Generation HIV-1 Based Vector: Efficiency and Role of Heparan Sulfate
Elena Copreni, Stefano Castellani, Lucia Palmieri, Salvatore Carrabino, Luigi Naldini, Massimo Conese

359 Specific Modulation of Tight Junctions to Enhance Airway Gene Transfer
Ruth S. Everett, Miriam K. Vanhook, Larry G. Johnson

360 Disturbance of Airway Epithelium Is Reduced, Whilst Lentivirus-Mediated Gene Transfer Is Maintained, after Low Dose Lysophosphatidylcholine Pre-Treatment In Vivo
Patricia Cmielewski, Alice G. Stocker, David W. Parsons, Donald S. Anson

361 Radiation-Induced Lung Apoptosis Is Mediated by TNF-alpha Action
Ming Zhang, Jun Qian, Ming Chen, Spring Kong, Theodore S. Lawrence

362 Using Real-Time (TaqMan®) PCR to Genotype Offspring of Transgenic CF-null Mice – A Core Facility of the UK Cystic Fibrosis Gene Therapy Consortium
Rebecca L. Smith, Lee A. Davies, Hazel Painter, Anusha Varathalingam, Deborah R. Gill, Stephen C. Hyde

363 Gene Delivery to Human Sweat Glands: A Model for Cystic Fibrosis Gene Therapy
Haeyul Lee, David R. Koehler, Cho Y. Pang, Ronald H. Levine, Philip Ng, Donna J. Palmer, Paul M. Quinton, Jim Hu

364 Prevention of Radiation Pneumonitis by Recombinant Adenovirus-Mediated Transfer of Soluble TGF-b1 Gene
Koichi Takayama, Haiping Zhang, Junji Uchino, Akiko Harada, Taishi Harada, Yôichi Nakanishi

365 Aerosol Delivery of an Enhanced Helper-Dependent Adenovirus Formulation to Rabbit Lung Using an Intratracheal Catheter
David R. Koehler, Helena Frndova, Kitty Leung, Emily Louca, Donna Palmer, Philip Ng, Colin McKerlie, Peter Cox, Allan L. Coates, Jim Hu

366 Development of Quantitative TaqMan RT-PCR for the Evaluation of Non-Viral Mediated Gene Transfer to the Airways
Ian A. Pringle, Rebecca L. Smith, Bryony L. Jones, Deborah R. Gill, Stephen C. Hyde
ABSTRACTS

THURSDAY, JUNE 2

367
Safety and Immunological Responses Following Intraperitoneal Administration of an AAV5 Vector Encoding Human α1-Antitrypsin to Non-Human Primates

Ben-Gary Harvey, Bishnu P. De, John P. Ayala, Neil R. Hackett, Adriana Heguy, Ronald Crystal

Gene Regulation: Gene Targeting and RNA

368
Differential Sensitivity of Normal and CML-Derived CD34+ Cells to Inhibition of SHP2, Gab2 and Stat5
Gene Expression by RNA Interference (RNAi)

Michaela Scherr, Karin Battner, Anushar Chaturvedi, Beate Schultheis, Arnold Ganser, Matthias Eder

369
Targeted Gene Modification Via Triple Helix-Forming Oligonucleotides

Peter M. Glazer, Faye Rogers, Janice Lloyd

370
Multi-Copy, Multi-siRNA Vectors as Versatile Tools for Multiple Gene Knock-Down Applications

Laura Poliseno, Monica Evangelista, Mauro Giacca, Giuseppe Rainaldi

371
Optimizing Custom Zinc-Finger Nucleases for Use in Human Cells

Stephen Alwin, Maja B. Gere, Eva Guhl, Karin Effertz, Carlos F. Barbas III, David J. Segal, Matthew D. Witzman, Toni Cathomen

372
Targeting of Non-Viral Vectors to Specific Subnuclear Domains

Joshua Z. Gasiorowski, David A. Dean

373
Use of Lentiviral and Adeno-Associated Vectors for Targeted Gene Inactivation in Human Cancer Cells

Sabrina Arena, Elisa Vigna, Luigi Naldini, Alberto Bardelli

374
Knockdown of Shp-2 Expression by siRNA in Mouse Embryonic Stem Cell Derived EB Cells Affects Their Hematopoietic Development

Gang-Ming Zou, Rebecca J. Chan, W. Christopher Shelley, Mervin C. Yoder

375
Targeted Gene Repair Induces the DNA Damage Response Pathways through the Activation of ATM, and the Stalling of Replication Forks Leading to a Blockage of Inherited Correction

Luciana Ferrara, Julia Engstrom, Erin Brachman, Miya Drury, Eric B. Kmiec

376
Various Aspects of the Single-Stranded DNA Fragment-Mediated Gene Correction

Hiroaki Tsujiya, Hideyoshi Harashima, Hiroaki Kamiya

377
Developing siRNAs to the E2Fs to Control Vascular Growth and Remodeling

Paloma H. Giangrande, JianXin Zhang, Julianna Layzer, Otto Hagen, Joseph R. Nevins, Bruce A. Sullenger

378
Retargeting Mobile Group II Introns to Repair Mutant Genes

Monique N. Kierlin, John P. Jones III, Rob G. Coon, Jiri Perutka, Alan M. Lambowitz, Bruce A. Sullenger

379
New Zinc Finger Protein Nuclease Architectures for More Efficient Gene Modification Therapies


380
Studies on the Mechanism of Gene Repair Using Saccharomyces cerevisiae as a Model System

Katie Maguire, Hetal Parekh-Olmedo, Eric B. Kmiec

381
Methods for Optimizing Lentiviral Vector-Mediated Gene Suppression by RNA Interference

Eddy Kizana, Genaro Ramirez, Eugenio Cingolani, Roselle Abraham, Eduardo Marban

382
Expanding the Repertoire of Heterospecific LoxP Sites for Cre-Mediated Site-Specific Recombination

Stephen J. Langer, Jarred Keith, Leslie A. Leinwand

383
Zinc Finger Nuclease-Boosted Gene Targeting & Synergistic Transient Regenerative Stem Cell Gene Therapy: Toward Clinical Gene Repair/Alteration & Custom Site-Specific Integrative Gene Therapy

Roger Bertolotti

384
Double-Stranded DNA Breaks Stimulate the Frequency of Targeted Gene Repair in Mammalian Cells

Timothy Schwartz, Luciana Ferrara, Hetal Parekh-Olmedo, Eric B. Kmiec

385
Gene Correction in the Absence of DNA Repair: A Universal Model Involving Homologous Pairing and DNA Replication

Hetal Parekh-Olmedo, Erin Brachman, Eric B. Kmiec
ABSTRACTS

FRIDAY, JUNE 3

Oral Abstract Session 330
RNA Virus Vectors: Integration and Vector Safety
Room: Ferrara Theater

386 2:00 PM
Large Scale Analysis of Foamy Virus Vector Integration Sites in Human CD34+ Cells
Grant Trobridge, Daniel G. Miller, Michael A. Jacobs, James M. Allen, Erik Olson, Hans-Peter Kiem, Rajinder Kaul, David W. Russell

387 2:15 PM
Myeloid Sarcoma Associated with Gene Marking of Hematopoietic Stem Cells in a Rhesus Macaque
Ruth Seggewiss, Stefania Pittaluga, Rima Adler, Cole Ferguson, Elio F. Vanin, Patrick F. Kelly, Robert E. Donahue, Brian P. Sorrentino, Arthur W. Nienhuis, Cynthia E. Dunbar

388 2:30 PM
Use of the cHS4 Chromatin Insulator to Reduce Oncoretrovirus Vector-Mediated Genotoxicity
David W. Emery, Changlong Li, George Stamatoyannopoulos

389 2:45 PM
Testing the Oncogenic Potential of Retroviral and Lentiviral Vector Integration
Eugenio Montini, Daniela Cesana, Francesca Sanvito, Sergi Sergi Lucia, Benedicenti Fabrizio, Ponzoni Maurilio, Claudio Doglioni, Luigi Naldini

390 3:00 PM
Flanking a Retrovirus Vector with the cHS4 Chromatin Insulator Reduces the Frequency of Vector-Mediated Trans-Activation of Endogenous Genes
Changlong Li, David W. Emery

391 3:15 PM
Effective Gene Therapy with Non-Integrating Lentivirus Vectors

392 3:30 PM
Development of a New Lentiviral Vector for Site-Specific Integration into Mammalian Genomes
Angelo Lombardo, Michelle Calos, Luigi Naldini

393 3:45 PM
Site-Directed Integration in Human Genome by Lentiviral Vectors Containing HIV-1 Integrase/Polydactyl Zinc Finger Fusion Proteins
Wenjie Tian, Zheng Dong, Vivian Luo, Sanson A. Chow

394 4:00 PM
Oncogenesis Following Delivery of a Non-Primate Lentiviral Gene Therapy Vector to Fetal Mice

395 4:15 PM
Safety of In Utero Gene Delivery of Lentiviral Vectors
Susan M. Kingsman, James E. Miskin, Pippa A. Raddiffe, Caroline V. Hacker, Daniel Chipchase, Nicholas D. Mazarakis, Kyriacos A. Mitrophanous

Oral Abstract Session 331
AAV Vectors: Vector Development
Room: 240 Complex

396 2:00 PM
Identification and Characterization of Novel AAV Isolates
Michael K. Schmidt, Emmanuelle Grott, Charles Buck, John A. Chiorini

397 2:15 PM
AAV-Mediated Gene Transfer to Endothelial Cells
Mo-Ying Hsieh, Yi-Lin Liu, Alexander Schlachterman, Katherine A. High

398 2:30 PM
Adeno-Associated Virus Serotype 8 Efficiently Crosses Blood Vessel Barrier in Muscle and Heart for Whole-Body Gene Delivery
Zhong Wang, Tong Zhu, Chumping Qiao, Liqiao Zhou, Bing Wang, Jian Zhang, Chunlian Chen, Juan Li, Xiao Xiao

399 2:45 PM
rAAV2 Movement Through the Late and Recycling Endosomes Is Influenced by Titer of Infection
Liang Zhang, Wei Ding, John F. Engelhardt

400 3:00 PM
Successful Production of Pseudotyped Mosaic rAAV Vectors Using a Modified Baculovirus Expression System
Erik Kohlbrenner, George Aslanidi, Kevin Nash, Stanislav Shklayev, Martha Campbell-Thompson, Barry J. Byrne, Richard O. Snyder, Nicholas Muzychka, Kenneth H. Warrington, Sergei Zolotukhin

401 3:15 PM
Evaluation of Primitive Murine Hematopoietic Stem and Progenitor Cell Transduction In Vitro and In Vivo by Recombinant Adeno-Associated Virus Vectors Based on Serotypes 1 through 5
Li Zhong, Weiming Li, Yanjun Li, Keyun Qing, Mervin C. Yoder, Ann Srivastava
ABSTRACTS

FRIDAY, JUNE 3

402 3:30 PM
Comparison of Pseudotyped rAAV Vectors for Transduction of Primary Hematopoietic Stem and Progenitor Cells Assayed In Vitro and In Vivo
Lijing Li, Sarah Hardy, Leslie Smith-Powell, Sai Srinivas, Stephen J. Forman, K. K. Wong, Saswati Chatterjee

403 3:45 PM
Defined Vector Substrates Used To Probe rAAV DNA Integration
Douglas M. McCarty, Vivian Choi, Alda Fernandes, R. J. Samulski

404 4:00 PM
Roles of Inverted Terminal Repeats (ITRs) and Capsid Proteins from Novel NHP AAVs in rAAV Mediated Gene Transfer
Xiaoyang Zhou, Guangping Gao, Roland A. Owens, You Lu, Roberto Calcedo, Jim Miller, James M. Wilson

405 4:15 PM
Comparison Study of Structural Determinants for AAV Liver Transduction by Domain Swapping between AAV-2 and AAV-8 Capsids
Xuan Shen, Hui Xu, Zan Huang, Theresa A. Storm, Mark A. Kay

Oral Abstract Session 332
Adenovirus Vectors: Innate Immunity
Room: 120/124

406 2:00 PM
Role of MyD88 Signaling Pathway in the Host Immune Responses in Adenoviral Vector-Mediated Gene Therapy
Zhe Zhang, Guang Ping Gao, Lee M. Wetzler, Yan Zhi, James M. Wilson

407 2:15 PM
Complement Blockade Prevents Several Innate Toxicities Rapidly Induced after Intravenous Adenovirus (Ad) Vector Administration
Anne Kiang, Zachary Hartman, Jun-Ping Wei, Ruth Everett, Delila Serra, Haixiang Jiang, Michael M. Frank, Andrea Amalfitano

408 2:30 PM
The Kupffer Cell Scavenger Receptor Is Accountable for Hepatic Sequestration of Adenovirus
Hidde J. Haisma, Anna Rita Bellu, Marianne G. Rots, Gera Kamps, Josse Plantinga, Jan Kamps

409 2:45 PM
Identifying Functional Adenovirus-Host Interactions Using Tandem Mass Spectrometry
Anuj Gaggar, Martin Sadilek, Andre Lieber, Dmitry M. Shayakhmetov

410 3:00 PM
Adenovirus Activates Mouse Platelets and Induces Platelet Leucocyte Association
Maha Othman, Andrea Labelle, David Lillicrap

411 3:15 PM
Trapping of Adenovirus Vectors in Blood
Daniel Stone, Shaoheng Ni, Zong-Yi Li, Dmitry M. Shayakhmetov, Andre Lieber

412 3:30 PM
Renal Pathophysiology after Systemic Administration of a Recombinant Adenovirus: Changes in Drug Metabolism Based upon Vector Dose
Michael P. Boquet, Hong T. Le, Erin A. Clark, Shellie M. Callahan, Maria A. Croyle

413 3:45 PM
Functional Differences of the Reticuloendothelial System in C57BL/6 and Balb/c Mice Mediate Differential Effects of Kupffer Cell Blockade, Rag-Deficiency and Splenectomy on Transgene Expression after Adenoviral Transfer
Jan Snoeys, Geert Mertens, Joke Lievens, Desire Collen, Erik Biessen, Bart De Geest

414 4:00 PM
Vitamin B2: A Key Component for Controlled Inactivation of Viruses Suitable for Biological Use
Shellie M. Callahan, Piyanuch Wonganan, Linda J. Obenauer-Kutner, Suganto Sutjipto, Hong T. Le, Maria A. Croyle

415 4:15 PM
Evaluating Polyethylene Glycol (PEG) Modified Adenoviral Vectors for Cell Targeting and Detargeting and Reducing Vector Toxicity
Hoyin Mok, Michael A. Barry

Oral Abstract Session 333
Inborn Errors of Metabolism - Liver and Storage Disorders
Room: 260/264

416 2:00 PM
Glucose-Dependent Insulin Production by Liver-Specific, Glucose-Regulatable Synthetic Promoters Results in the Cure of Diabetes
Jaeseok Han, Brienne McLane, Hee-Sook Jun, Ji-Woon Yoon

417 2:15 PM
Meganuclease-Mediated Chromosomal Surgery in Living Animals
Agnes Gouble, Julianne Smith, Christophe Perez, Valerie Guyot, Jean-Pierre Cabaniols, Sophie Leduc, Laurence Fiette, Patrick Ave, Beatrice Micheaux, Philippe Duchateau Frederic Paques
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418 2:30 PM
rAAV-Mediated Expression of Adiponectin Receptor (AdipoR2) cDNA in Muscle Prevents the Development of Obesity in DIO Rats
George Aslanidi, Stanislav Shklayev, Erik Kohlbrenner, Vadim Kroutov, Glenn Walter, Martha Campbell-Thompson, Sergei Zolotukhin

419 2:45 PM
Generation of Transgene Product-Specific Regulatory CD4+CD25+ T Cells by Hepatic AAV Gene Transfer
O. Cao, E. Dobrzenski, B. L. Mingle, L. Wang, R. W. Herzog

420 3:00 PM
Tropism-Modified Adeno-Associated Virus Vector Mediates Targeting of Brain Vascular Endothelium In Vivo
Yong Hong Chen, Beverly L. Davidson

421 3:15 PM
Liver Contains Sufficient Sulfatase Modifying Factor for 4-Sulfatase and Correction of MPS VI with Neonatal Retroviral Gene Therapy in Cats
Mark Haskins, Thomas O’Malley, Bin Wang, Jason Metcalf, Ping Wang, John Hopwood, Katherine P. Ponder

422 3:30 PM
Dominant-Negative Interference in the Pahenu2 Mouse Model of PKU: Effectiveness of Vectors Expressing Either Modified Forms of Phenylalanine Hydroxylase (PAH) or Ribozymes Plus a Hardened PAH mRNA
Catherine E. Charron, O. P. Perera, Stacy L. Porvasnik, Mandy Blackburn, Alfred S. Lewin, Philip J. Laipis

423 3:45 PM
Increased Incidence of Hepatocellular Change and Neoplastic Disease in AAV-WPRE Treated Pahenu2 Mice
Jennifer E. Embury, Catherine E. Charron, Philip J. Laipis

424 4:00 PM
Intracranial AAV5 Synergizes with Non-Myeloablative Bone Marrow Transplantation in the Murine Model of Globoid-Cell Leukodystrophy
Darshong Lin, Anthony Donsante, Shannon Macauley, Beth Levy, Carole Vogler, Mark Sands

425 4:15 PM
AAV2- and AAV5-Mediated CNS Delivery of Human Aryl Sulfatase A (hARSA) Prevents Sulfatide Storage and Neuropathological Phenotype in Metachromatic Leukodystrophy (MLD) Mice
Nathalie Cartier, Caroline Sevin, Abdellatif Benraiss, Peter DeDeyn, Delphine Bonnin, Marie-T Vanier, Moullier Philippe, Völkmar Gieselmann, Patrick Aubourg

426 2:00 PM
Cure of Established Disease and Direct Evidence of Cellular Cross-Correction in the Nervous System of Metachromatic Leukodystrophy Mice after HSC-Based Gene Therapy
Alessandra Biffi, Alessia Capotondo, Angelo Quattrini, Ubaldo Del Carro, Riccardo Brambilla, Stefania Fasano, Sergio Marchesini, Claudio Bordignon, Luigi Naldini

427 2:15 PM
AAV2- and AAV5-Mediated CNS Delivery of Human CLN2 Reduces Lysosomal Storage in a Mouse Model of Late Infantile Neuronal Ceroid Lipofuscinosis

428 2:30 PM
A Novel Approach for the Treatment of the Neurological Symptoms of MPS VII (Sly Disease)
Brian J. Spencer, Inder M. Verma

429 2:45 PM
AAV5-Mediated Delivery of Human Aryl Sulfatase A (hARSA) Prevents Sulfatide Storage and Neuropathological Phenotype in Metachromatic Leukodystrophy (MLD) Mice
Nathalie Cartier, Caroline Sevin, Abdellatif Benraiss, Peter DeDeyn, Delphine Bonnin, Marie-T Vanier, Moullier Philippe, Völkmar Gieselmann, Patrick Aubourg

430 3:00 PM
Immune System Regulation of Transgene Expression from High-Capacity Gutless Adenoviral Vectors in the Mouse Brain: Very Long Term Expression Following Pre-Immunization
Carlos Barcia, Jeffrey M. Zinger, Weidong Xiong, Philip Ng, Donna Palmer, Maria G. Castro, Pedro R. Lowenstein

431 3:15 PM
In Utero CNS Delivery of an Integrating HSV-1 Amplicon Vector Leads to Prolonged, Neuron-Specific Gene Expression
William J. Bowers, Michael A. Mastrangelo, Ann E. Casey, Hilary A. Southerland, Kathleen A. Maguire-Zeiss, Howard J. Federoff

432 3:30 PM
Molecular Chimerism Prevents Experimental Autoimmune Encephalomyelitis (EAE)
Herena Eixarch, Carmen Espejo, Francisco Vidal, Silvia Garces, Mireia Castillo, Alex Bote, Elisabeth Kadar, Marta Rosal, Xavier Montalban, Jordi Barquinero
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433 3:45 PM
Gene Therapy of Epilepsy by Adenovirus-Mediated Tetanus Toxin Light Chain Gene Transfer
Jun Yang, Qingshan Teng, Mary E. Garrity-Moses, Thais Federici, Imad Najm, Stephan Chabardes, Michael Moffitt, Nicholas M. Boulis

434 4:00 PM
Long-Term Effects on Retinal Function and Structure Using AAV-Mediated Gene Therapy in Blind RPE65 Null Mutation Dogs
Kristina Narfstrom, Greg Tillis, Ragheidur Bragadottir, Mathias Seeliger, Elizabeth P. Rakoczy, Martin L. Katz

435 4:15 PM
Functional and Morphological Rescue of the Type I Ocular Albinism Murine Retina Following AAV-Mediated Gene Transfer.
Enrico M. Surace, Luciano Domenici, Katia Cortese, Consuelo Venturi, Gabriella Catugno, Umberto Di Vicino, Alessandro Cellerino, Valeria Marigo, Carlo Tacchetti, Andrea Ballabio Alberto Auricchio

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Infectious Diseases and Vaccines: RNAi in Gene Therapy for Infections
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436 2:00 PM
shRNAs Target HIV-1 Vif Gene Coding for SOCS-Box Motif Prevent Virus Escape from RNA Interference-Mediated Inhibition
Jianfei Ji, John J. Rossi

437 2:15 PM
Alternative Strategies To Deliver Multiple shRNAs in Order to Simultaneously Inhibit Multiple Targets
Peter W. Roelvink, Michael W. Graham, David A. Suhy, Linda B. Couto, Sara M. Cunningham, Mark A. Kay, John J. Rossi, Alexander A. Kolykhalov

438 2:30 PM
Development of an siRNA Based Therapy for Hepatitis Virus Infection

439 2:45 PM
CXCR4 and CCR5 shRNA Transgenic Macrophages Are Functionally Normal and Resist HIV-1 Infection
Joseph Anderson, Ramesh Akkina

440 3:00 PM
A Non-Human Primate Model for Lentivirus-Mediated Anti-HIV RNAi Strategies
Karen Beagles, Grant Todorov, Brian Beard, John Ross, Jiing-Kuan Yee, Shiu-Lok Hu, Hans-Peter Kiem

441 3:15 PM
Short Hairpin RNA Interference Expressed from a Gene-Deleted Adenoviral Vector Results in Reduction of Hepatitis B Surface Antigen Levels In Vitro and in a Small Animal Model for Hepatitis B Infection
Anja Ehrhardt, Hui Xu, Felix H. Salazar, Patricia L. Marion, Mark A. Kay

442 3:30 PM
Evaluation of siRNA for Induction of In Vitro Resistance in HIV-1
Priscilla Yam, Jerry Wu, Ying Yu, John A. Zaia, Jiing-Kuan Yee

443 3:45 PM
Tissue Specific Expression of Short Hairpin RNAs for the Treatment of HCV Using Liver Specific RNA Pol II Expression Cassettes
David A. Suhy, Alexander A. Kolykhalov, Linda B. Couto, Luz Maria Garcia, A. Rosanna Schroeder, Amy E. Parker, Gabriel Haniff, Sara M. Cunningham, Mark A. Kay, Peter W. Roelvink

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Targeting of Adeno Viral Vectors and Cancer Treatment
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444 2:00 PM
A New Vector Which Targets Breast Cancer and Its Vasculature
Yanzheng Liu, Tao Ye, Jonathan Maynard, Hakan Akbulut, Albert Deisseroth

445 2:15 PM
Combinatorial Antiangiogenic Gene Therapy by Nonviral Gene Transfer Using the Sleeping Beauty Transposon Causes Tumor Regression and Improves Survival in Mice Bearing Intracranial Human Glioblastoma

446 2:30 PM
Tie2 Expression Defines an Integrated System of Cell Types Specifically Involved in Angiogenesis, and Provides a Platform for Targeted Gene Delivery to Tumors
Michele De Palma, Mary Anna Venneri, Rosella Galli, Nathalie Belmonte, Maurilio Sampaolesi, Lucia Sergi Sergi, Luigi Naldini
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447 2:45 PM
Mesenchymal Stem Cells as Delivery Systems for Cancer Therapy: Evaluation of Tropism and Efficacy
Frank Marini, Jennifer Dembinski, Matus Studeny, Claudia Zompetta, Michael Andreeff

448 3:00 PM
A Genetic Strategy to Inhibit Complement Activation Arising from Adenoviral Vectors
Kurt R. Zinn, Hongju Wu, Amanda Stargel, April J. Adams, Tandra R. Chaudhuri

449 3:15 PM
Tumor Targeted, Systemic Delivery of Therapeutic Viral Vectors Using Hitch Hiking on Antigen Specific T Cells
Jian Qiao, Caroline Cole, Timothy Kotke, Rosa Maria Diaz, Luis Sanchez-Perez, Gregory Brunn, Jill Thompson, Richard Vile

450 3:30 PM
Combination of Fas Gene Therapy and Ceramide Analogues: A Double Edge Sword for the Treatment of Head and Neck Cancer
Saeed N. Elojeimy, John C. McKllap, Ahmed M. El-Zawahry, David A. Schwartz, David H. Holman, Xiang Liu, Terry Day, Alicja Bielauskas, Yujsf A. Hamann, James S. Norris

451 3:45 PM
Matrix Metalloproteinase-Targeted Oncolytic Sendai Virus Vector “Armed” with a Suicide Gene “Yeast Cytosine Deaminase”: Remarkable Combinational Effects
Hiroyuki Kinoh, Makoto Inoue, Kentaro Washizawa, Eiji Akiba, Mamoru Haozawa

452 4:00 PM
The Enhancement of Anticancer Effect of Chemotherapy by the Delivery of Rad51 siRNA
Makoto Ito, Seiji Yamamoto, Keisuke Nimura, Katsuo Tamai, Yasufumi Kaneda

453 4:15 PM
The Histone Deacetylase Inhibitor FK228 Can Increase Adenovirus Transgene Protein Expression in Human LOX IMVI Melanoma Xenografts
Merrill E. Goldsmith, Alian Aguila, Michael C. Alley, William R. Wand, Susan Bates, Tito Fojo

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Advances in Hematologic Gene Therapy
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454 2:00 PM
Mouse Models for the Analysis of Genetic Risk Factors for Hematopoietic Transformation in XSCID Gene Therapy
Yan Shou, Lilia Stepanova, Brian P. Sorrentino

455 2:15 PM
Drug Resistance Gene Therapy to Induce Donor-Specific Tolerance after Nonmyeloablative Allogeneic Stem Cell Transplantation in Dogs
Sabine Gerull, Karen E. Beagles, Brian C. Beard, Laura J. Peterson, Hans-Peter Kiem

456 2:30 PM
HOXB4 Overexpression Expands Short-Term Repopulating Cells in Nonhuman Primates and Dogs
Xiao-Bing Zhang, Laura J. Peterson, Alexanadra A. Knapp, Brian C. Beard, R. Keith Humphries, Hans-Peter Kiem

457 2:45 PM
The Benefits of CH-296 during Ex Vivo Gene Transfer Go beyond the Ability to Co-Localize the Retroviral Particle and Target Cell
Mo A. Dao, Ian A. Nolta

458 3:00 PM
Reversal of Canine Leukocyte Adhesion Deficiency by Retroviral-Vector Mediated Gene Therapy with Non-Myeloablative Conditioning
Thomas R. Bauer, Jr., Laura M. Tischong, Mehreen Hai, Yuchen Gu, Robert A. Sokolic, Tanya Burkholder, John D. Bachr, Dennis D. Hickstein

459 3:15 PM
Long-Term Treatment of Canine Cyclic Neutropenia by G-CSF-Lentivirus
William Osborne, Margaret Brzezinski, Ofer Yanay, Lanaya Waldon, Jeffrey Christensen, Denny Liggitt, David Dale

460 3:30 PM
Transplantation of Human Aldehyde Dehydrogenase Expressing Cells Leads to Widespread Tissue Distribution of Donor Cells in the Pancreas and Liver of NOD/SCID/MPVII Mice
David A. Hess, Timothy P. Craft, Louisa Wirthlin, Phillip E. Herrbrich, Alex A. Hofling, Mark S. Sands, Jan A. Nolta

461 3:45 PM
Qualitative and Quantitative Luciferase Expression In Vivo of Transduced Hematopoietic Cell populations Using Bioluminescent Imaging (BLI)
Henk Rozemuller, Ingrid F. H. Koggel, Robbert Spaapen, Anton Hagenbeek, Anton C. M. Martens

462 4:00 PM
Busulfan Dose Escalation to Increase Gene Marking of Hematopoietic Stem Cells by Lentiviral Vectors in Infant Rhesus Monkeys
Christoph A. Kahl, Alice F. Tarantal, Chang I. Lee, Daniel F. Jimenez, Christopher Choi, Karen Pepper, Denise Peterson, Misty D. Fletcher, Alyssa C. Leapley, Donald B. Kohn
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Efficient Lentiviral Gene Transfer and Expression in Human Embryonic Stem Cells
Carolyn Lutzko, Xiaojin Yu, Dinithi Senadheera, Donald B. Kohn

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Gene Regulation: Regulated Systems and Cell Engineering
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464 2:00 PM
Development of a Novel Gene Regulation System Downstream of Promoter
Hongzhe Duan, Chengwen Li, Richard Jude Samulski

465 2:15 PM
In Vivo Selection of Genetically Modified Primary Human Hematopoietic Cells Using a Cell Growth Switch
Lindlin Wang, Yasuo Nagasawa, Brent Wood, Ingrid Lintmaer, Thalia Papayannopoulou, Michael Harkey, Cynthia Nougirat, C. Anthony Blau

466 2:30 PM
Long-Term Rapamycin Control of Transgene Expression in Mouse Salivary Glands in a Single AAV Vector
Jianghua Wang, Antonis Voutetakis, Milton Papa, Victor M. Rivera, Tim Clackson, Beatrijs M. Lodde, Fumi Mineshiba, Bruce J. Baum

467 2:45 PM
Allogeneic Bone-Marrow Transplantation in Mice Provides Tolerance and Promotes Massive In Vivo Selection of Subsequently Transplanted Hepatocytes
Konrad L. Streetz, Regis Doyonnas, Denny D. Jenkins, Seppi Lin, Judith A. Shizuru, Helen Hanenberg, Mark A. Kay

468 3:00 PM
Cellular Therapy with Transgene Expressing APC Activates CD4+CD25+ Regulatory T Cells Which Modulate the Immune Response to Gene Therapy Derived Products in Immunocompetent Mice
Andrea Annoni, Manuela Battaglia, Antonio Follenzi, Angelo Lombardo, Luigi Naldini, Maria Grazia Roncarolo

469 3:15 PM
Induction of Donor Specific Tolerance Using Gene Therapy and Sublethal Conditioning
Daron Forman, Chaorui Tian, John Iacomini

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Foamy Viral Mediated Transduction of Recombinant FANCC in the Absence of Prestimulation Is Sufficient To Restore the Long Term Repopulating Activity of Fancc -/- Stem Cells and Prevent the Acquisition of Myeloid Malignancies
Yue Si, Cordula Leurs, Edward F. Stour, Jin Yuan, Helmut Hanenberg, D. Wade Clapp

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Microvessel Injury and Irradiation Damage in Salivary Glands
Ana Paola P. Cotrim, Jianghua Wang, Antonis Voutetakis, Anastasia L. Sowers, James B. Mitchell, Bruce J. Baum

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Hematopoietic Cell Differentiation from Common Marmoset (Callithrix jacchus) Embryonic Stem Cells by Their Genetic Manipulation Using the Third Generation Lentiviral Vector
Tomoko Yokoo, Ryo Kurita, Erika Sasaki, Takashi Hiyoyama, Yukoh Nakazaki, Kiyoko Izawa, Hajime Ishii, Yoshikuni Tanioka, Kisaburo Hanazawa, Yuan Son Bai Yasushi Soda, Kenzaburo Tani

3:30 PM
Transduction of Minimally Stimulated Hematopoietic Progenitor Cells with a Foamy Virus Vector That Expresses MGMTP140K
Shanbao Cai, Jennifer R. Hartwell, Aaron G. Ernstberger, W. S. Goebel, Helmut Hanenberg, Karen E. Pollok

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In Vivo Targeting of MLV(HIV) Pseudotype Vectors to T Lymphocytes in a Transgenic Mouse Model
Silke Schuele, Stefanie Steidl, Sylvia Raupp, Cheick Coulibaly, Ulrich Kalinke, Klaus Cichutek, Matthias Schweizer

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HIV-1 and HIV-2 Chimeric Lentiviral Vectors for Gene Transfer
Geetanjali Sachdeva, Kritika Kachapati, Suresh Arya
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477  Development of SIV Based Vectors with Efficient Delivery for Vector Dynamic Studies in the Simian System  
Christopher Chen, Jiamo Lu, Tatiana Slepushkina, Andrew Worden, Vladimir Slepushkin, Laurent M. Humeau, Gwendolyn K. Binder, Xiaobin Lu

478  A HIV-1 Based Cross-Packaging System for FIV Vectors  
Adam S. Cockrell, Hong Ma, Thomas McCown, Kim D. Kluckman, Randy J. Thresher, Tal Kafri

479  Targeted Entry of Retroviral Vectors Depends on Characteristics of the Target Receptor and the Chimeric Envelope Protein  
Lorraine M. Albritton, Fang Li, Pankaj Kumar, Langzhu Tan

480  Examination of the Relationship between the Level of Gene Transfer and the Number of Envelope Proteins That Are Incorporated Per Retrovirus Particle When High Doses of Purified Virus Stocks Are Used to Transduce Cells  
Natalia Landázuri, Joseph M. Le Doux

481  Dynamics of Transduction of Human Cells by EIAV Vectors – The Importance of Cell Entry  
Daniel C. Farley, Fraser J. Wilkes, James E. Miskin, Susan M. Kingsman, Kyriacos A. Mirophanous

482  Neuronal Gene Transfer with HIV-1-Based Lentiviral Vectors Pseudotyped with Lyssavirus Glycoproteins  
Qingshan Tang, Mary E Garrity-Moses, Thais Federici, Jun Yang, Robert Kutner, Noel Tordo, Jakob Reiser, Nicholas M. Boulis

483  Targeted Receptor Trafficking Affects the Efficiency of Retrovirus Transduction  
Delfi Krishna, Julia Raykin, Joseph Le Doux

484  Ultrasound-Mediated Microbubble Destruction and Retroviral Gene Delivery  
Sarah L. Taylor, Ahad Rahim, Nigel Bush, Jeff Bamber, Colin Porter

485  Lentivirus-Mediated Gene Transfer to Cultured Fetal Sheep Airway Epithelium and Lung Tissue Explants  
Ze-Yan Yu, Karen McKay, Peter Van Asperen, Maoxin Zheng, Jane Fleming, Samantha Ginn, Eugenie Lumbers, Peter Rowe, Ian Alexander

486  Optimized Conditions for Retroviral Gene Transfer by RetroNectin® Bound Virus Infection Method  
Hideto Chono, Hiromi Okayama, Yasushi Katayama, Nobuto Kayama, Junichi Mineno, Kiyozo Asada, Ikumoshin Kato

487  Enhancement of Adeno- and Lentivirus Mediated Gene Transfer in Human Tumor Cell Lines by Cell Permeable Peptides and Polycations  
Saatu Lehmusvuara, Outi Meriläinen, Tanja Hakkarainen, Jarmo Walijöös

488  Engineering Enhanced Retroviral Vectors through the Insertion of a His, Tag into VSV-G  
Julie H. Yu, David V. Schaffer

489  Further Studies of the Most Efficient Vector Systems for Gene Transduction into Human Dendritic Cells Line (im-NMD)  
Eigo Sato, Masanari Kato, Yuku Hara, Hua Yan, Tohko Miyagi, Xiao-Kang Li, Wataru Sugihara, Naoki Yamamoto, Kenichi Teramoto, Shigeaki Arii Hiromitsu Kimura

490  Lentiviral-Mediated Gene Transfer to Cells of the Vasculature  
Ciara A. O'Shea, Robert J. Nelson, Padraig Strappe, Timothy O'Brien

491  Development of Fast and Efficient Methods for the Purification and Concentration of Lentiviral Vectors  
Rachel M. Koldej, Donald S. Anson

492  Harvest Timing and Media Composition Effects on Lentiviral Vector Production  
Christopher Ballas, Terry Johnson, Kenneth Cornetta

493  Concentration and Purification of Lentivirus Pseudotypes Bearing Alternative Envelope Glycoproteins  
Robert H. Kutner, Jakob Reiser

494  Assessing Lentiviral Vector Quality Using Transmission Electron Microscopy  
Lakshmi Sastry, Carole Miller, Terry Johnson, Aparna Jasti, Vince Gattone, Kenneth Cornetta

495  Upscaling of Retroviral Vector Production under GMP Guidelines to Generate High Titer Preparations for Clinical Use  
Sandra Jung, Peter Leyendecker, Frank Kogelberg, Christian Wölf, Andreas Schilz, Klaus Kuehleke, Sonja Naundorf
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Stability of Oncolytic Measles Viruses during Storage and Clinical Preparation
Troy R. Wegman, Kirsten K. Langfield, Henry J. Walker, Gay E. Griesmann, Julie A. Sauer, Sharon A. Stephan, Mark J. Federspiel

AAV Vectors: Vector Biology

497
Sex and Estrous Cycle Stage Influence the Efficiency of AAV-Mediated Gene Transfer in the Rodent Brain
James C. Dodge, Jennifer Clarke, Marco A. Passini, Anthony Song, Catherine R. O’Riordan, Seng H. Cheng, Gregory R. Stewart

498
Adeno-Associated Virus Type-1 Vectors Efficiently Transduce Human Polarized Airway Epithelia without Polarity Bias
Ziying Yan, Xiaoming Liu, Chi-Man Lei, Meihui Luo, John F. Engelhardt

499
Species-Specific Differences in the Polarity of rAAV5 and rAAV2 Transduction of Mouse and Human Airway Epithelia
Xiaoming Liu, Ziying Yan, Meihui Luo, John F. Engelhardt

500
Biology of Novel AAV Serotype Vectors in the Liver Directed Gene Transfer to Nonhuman Primates
Guangping Gao, You Lu, Roberto Calcedo, Rebecca Grant, Julio Sanmiguel, Lili Wang, Julie Johnston, Joanita Figueredo, Martin Lock, James M. Wilson

501
AAV6 Pseudotyped Double-Stranded-AAV Vectors Allow Adenoviral-Like Transduction and Brute-Force Integration in the Growing Mouse
Ognjen Petras, Timothy J. Davern, Marcus O. Muench, Xiaodong Han

502
GFP-Tagged Adeno-Associated Viral (AAV) Particles Allow the Study of Cytosolic and Nuclear Trafficking
Kerstin Lux, Stefanie Schlemmer, Luca Perabo, Daniela Goldnau, Jan Eindell, Michael Hallek, Hildegard Buening

503
Mutational Strategy To Enhance or Rescue Functionality of Existing AAV Vectors
Luk H. Vandenberghhe, Michael H. Yang, Julie Johnston, Guangping Gao, James M. Wilson

504
In Vivo Characterization of AAV Serotype 9 Vectors in Mice
Katsuya Inagaki, Theresa A. Storm, Sally Fuess, Mark A. Kay, Hiroyuki Naka

505
Analysis of AAV Genome Conversion and Stabilization
Bernd Hauck, Jing Xie, Weidong Xiao

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Transductional and Genome Biodistribution of Novel AAV Serotype Vectors after Systemic Delivery and Their Liver Specific Dose Response Profiles in Mice
You Lu, Guangping Gao, Julio Sanmiguel, Roberto Calcedo, Peter Bell, Nelson Wivel, James M. Wilson

507
Modeling Adeno-Associated Virus 2 Capsid for the Identification of Targeting Ligands for Enhanced Gene Transfer to Polarized Human Airway Epithelial Cells
April E White, Marina Mazur, Eric J. Sorscher, Selvarangan Ponnazhagan

508
Impact of Sex on Serum α1-Antitrypsin Levels Following Intrapleural and Intravenous Administration of AAVrh.10 Vectors to Mice
Bishnu P. De, Adriana Heguy, Neil R. Hackett, Guangping Gao, James M. Wilson, Ronald G. Crystal

509
Endogenous Estradiol Augments RGD Modified AAV2-Mediated Gene Transfer in Human Ovarian Epithelial Cancer
Wenfang Shi, Jeffery Bartlett

510
Neonatal Gene Transfer with Non-Human Primate AAV Vector Serotypes Results in Widespread Transduction and Gene Activity in the Mouse Brain
Brian A. Karolewski, Hennessy Howell, John H. Wolfe

511
Effect of Hepatocyte Division on Molecular Fate of rAAV DNA
Young-Kook Choi, Yuanqing Lu, Sihong Song

512
The Cellular TATA-Binding Protein Is Required for Replication of a Minimal Adeno-Associated Virus Type 2 (AAV-2) p5 Promoter Element
Achille François, Mickael Guilbaud, Rafi Awedikian, Gilliane Chadeuf, Philippe Mouliier, Anna Salvetti

513
Novel Characteristics of Heparin Binding by Adeno-Associated Virus Serotype 6
James M. Allen, Eric E. Finn, Jeffrey S. Chamberlain
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Effect of DNA-PKcs on AAV Replication
Young-Kook Choi, Irene Zolotukhin, Barry J. Byrne, Sihong Song

515
Overlapping Adeno-Associated Viral (AAV) Vector Mediated Gene Transfer Is Dependent on Viral Serotype and the Transgene Sequence in Skeletal Muscle
Arkasubhra Ghosh, Yongping Yue, Dongsheng Duan

516
Analysis of AAV Serotype 8 Vector Integration in Normal and DNA-PKcs-Deficient Scid Mice by a Novel Strategy
Katsuya Inagaki, Xiaolin Wu, Sally Fuess, Theresa A. Storm, Mark A. Kay, Hiroyuki Nakai

517
Type 1 Rep52 Is Superior to Authentic Rep52 for Producing Recombinant Adeno-Associated Virus Type 5 in Insect Cells
Masashi Urabe, Takayo Nakakura, Hiroaki Mizukami, Akihiro Kume, Robert M. Kotin, Keiya Ozawa

518
Production of Recombinant Type 2 Adeno-Associated Virus in Bioreactors
Yves Durocher, Phuong Lan Pham, Gilles St-Laurent, Danielle Jacob, Brian Cass, Josephine Nalbantoglu, Amine Kamen

519
Selective Inactivation of Helper Adenovirus with High Hydrostatic Pressure for AAV-2 Vector Production
Joshua N. Leonard, David V. Schaffer, Peter Fersl, Antonio Delgado

520
The Effects of Transgene and Cassette Size on Recombinant AAV2 Production and Expression
Corinne M. Goldsmith, Antonis Voutetakis, Bruce J. Baum

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Spliceosome-Mediated RNA Trans-Splicing with rAAV Partially Restores CFTR Function to Polarized Human CF Airway Epithelial Cells
Xiaoming Liu, Meilin Lu, Liang N. Zhang, Ziyong Yan, Weiding Roman Zak, Gary S. Mansfield, Lloyd G. Mitchell, John F. Engelhardt

522
Enhanced Repair of Articular Cartilage Defects in Rabbits by Overexpression of Human FGF-2 Via rAAV-Mediated Gene Transfer
Magali Cucchiarini, Henning Madry, Chunyan Ma, Tanja Thurn, David Zurakowski, Dieter Kohn, Michael D. Menger, Stephen B. Trippel, Ernest F. Terwilliger

523
AAV Transcytosis through Barrier Epithelia and Endothelium
Giovanni Di Pasquale, John A. Chiorini

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Adenovirus Vectors: Applications

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Optimized Gene Transfer into Mesenchymal Stem Cells and Embryonic Stem Cells by Modified Adenovirus Vectors
Hiroyuki Mizuguchi, Kenji Kawabata, Fuminori Sakurai, Tomomi Sasaki, Teruhisa Yamaguchi, Takao Hayakawa

526
Conditionally Replicative Adenoviruses Designed to Restore Defects in the p53 Pathway for Effective Virotherapy of Cancer
Victor W. van Beusechem, Danielle A. M. Heideman, Jan E. Carette, Martin Scheffner, Ramon Alemany, Winald R. Gerritsen

527
Reductions in Tissue Transduction and Toxicity after Systemic Administration of Adenovirus Vectors Containing Fiber-Shaft Exchange in Combination with both CAR- and av Integrin-Binding Ablation
Naoya Koizumi, Hiroyuki Mizuguchi, Kenji Kawabata, Fuminori Sakurai, Yoshiteru Watanabe, Takao Hayakawa

528
Infectivity Enhancement of a Genetic Fiber Mosaic Adenovirus Vector in Ovarian Cancer Cells
Yuko Tsuruta, Larisa Pereboeva, Joel N. Glasgow, Yosuke Kawakami, David T. Curiel

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Oncolytic Virotherapy Employing flt-1 Driven mda-7/IL-24 Gene Delivery
Sergey A. Kaliberov, Lyudmila N. Kaliberova, Amy S. Petersen, Valentina Krendelchtchikova, Viktor Krasnykh, Donald J. Buchsbaum

530
Adenoviral and Hydrodynamic Gene Transfer of Replicating Episomes Result in Stable Transgene Expression after Partial Hepatectomy in Mice
Jan Snoeys, Désiré Collen, Bart De Geest
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Balloon Catheter-Mediated Hepatic Vein Delivery of a Viral Vector Mitigates Neutralization by Anti-Viral Antibodies and Results in Efficient Transduction of Rabbit Liver
Bradley L. Hodges, Kristin M. Taylor, Qiuming Chu, Samantha E. Scull, Rebecca G. Serrillo, Scott C. Anderson, Ronald K. Scheule

532
Effects of a New YB-1 Dependent Adenovirus (XVir) in Human Prostate Cancer Cell Lines and in a Xenograft Mouse Model
Klaus Mantwill, Nadia Koehler-Vargas, Alexandra Bernshausen, Uwe Treiber, Rudolf Hartung, Bernd Gansbacher, Per Sonne Holm

533
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Ramil E. Sapinoro, Angela P. H. Burgess, Stephen Dewhurst

534
Optimising Expression Cassettes for Vascular Gene Expression
Stephen J. White, Melanie A. Roberts, Jeremy R. Sanford, Javier F. Cáceres, Andrew C. Newby

535
Long-Term Correction of Hyperbilirubinemia in a Rat Model of Crigler-Najjar Syndrome Type 1
Viraj P. Mane, Gabriele Toetella, Wilma Norona, Milton Finegold, Phil Ng, Arthur L. Beaudet, Anthony F. McDonagh, Brendan Lee

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577 Distribution, Time Course, In-Vivo Imaging, Expression and Inflammatory Response after Non-Viral Gene Delivery to Rat and Primate CNS James G. Hecker, Ellen S. Hauck, Michael H. Nantz, Alice F. Tarantal

578 Quantitative Evaluation of the Relationship between Efficiency of Nuclear Transfer of Plasmid DNA and Trans-Gene Expression in Individual Cell: Consideration of Mechanism Underlying in the Heterogeneity of the Trans-Gene Expression Via Artificial Vector Hidetaka Akita, Rie Ito, Hiroyuki Kamiya, Kentaro Kogure, Hideyoshi Harashima

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604 Long-Term, Erythroid-Specific Expression of Human Factor IX in Hemophilic Mice Engrafted with Lentiviral Vector-Transduced Hematopoietic Stem Cells
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605 Efficient AAV 1 and AAV2 Hybrid Vector for Gene Therapy of Hemophilia
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607 Long-Term Phenotypic Correction of Canine Hemophilia B Following Systemic Administration of Helper Dependent Adenoviral Vector
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608 Cytotoxic T Lymphocyte Response to AAV2- and AAV8-Mediated Human Factor IX Gene Therapy in Mice
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624 Development of a Novel Ribbon-type NF-kB Decay Oligodeoxynucleotides to Treat Cardiovascular Diseases
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625 Adenoviral-Mediated Gene Delivery of a Constitutively Active Form of Early Growth Response Factor-1 Increases Tissue Perfusion in a Murine Model of Hindlimb Ischemia
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626 Ultrasound-Triggered/Enhanced Delivery of Oligonucleotides into Aorta
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627 Adenoviral-Mediated Systemic Transduction of the Angiotensin II (Ang II) Type 2 Receptor (AT2R) Enhances the Hypotensive Action of Losartan
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628 Adenovirus-Mediated Expression of b3-Adrenergic Receptor Kinase C-Terminus Reduces Intimal Hyperplasia and Luminal Stenosis in a Pig Model of Arteriovenous Polytetrafluoroethylene Graft Failure
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629 Successful Gene Therapy Against Atherosclerosis by AAV Delivery of IL-10 and TGFβ3ACT
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630 In Vivo hVEGF165 Gene Transfer Improves Early Endothelialisation and Patency in Synthetic Vascular Grafts
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632 Adenoviral Mediated Nuclear Factor kappa B Super Repressor Gene Transfer to Rat Insulinoma Cells Protects Against Cytokine Induced Oxidative Damage
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633 Synergistic Effects of Naked DNA Vectors Encoding VEGF and bFGF on Angiogenesis and Blood Flow Recovery in Ischemic Hindlimb
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634 Proliferation and Angiogenesis in Vascular Cells Overexpressing NOS Isoforms
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635 Experimental Study on Changes of the Revascularization and Expression of VEGF and Its Receptors in Ischemic Skeletal Muscle
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Development of a Selectable HSV Replication System Based on Calcium Influx Cytotoxicity Mediated by Expression and Activation of TRPV1
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652 Adeno-Associated Vector Delivery of Gutamate Transporters in an ALS Mouse Model
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653 Injection of AAV Directly into the Spinal Cord Increases Levels of Gene Expression
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654 Quantifying GDNF Levels in the Spinal Cord after Intracerebral Delivery of rAAV5 GDNF
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655 Compacted DNA Nanoparticles Effectively Transfect Brain Cells In Vitro and In Vivo
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656 Development of Viral Vectors To Mediate Neuron-Specific Gene Silencing
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657 A Nucleic Acid-Based Transgene Reporter for In Vivo MRI
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658 Development and Evaluation of an Infusion Device for AAV-Mediated Gene Delivery to the Brain
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659 The Plasticity of Hematopoietic Stem Cells (HSC): Rat HSC Transduced In Situ by rSV40 Vectors Differentiate into Multiple Lineages of CNS Cells
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660 Quantitative Comparison of AAV Serotypes AAV2, AAV5, and AAVrh.10 Efficiency for CNS Gene Therapy Following Intracranial Gene Delivery
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661 Efficient SV40-Mediated Gene Transfer to the Brain
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662 The Tracking of Exogenous Cells in the Injured Central Nervous System: Viral Vector Labeling Versus an Intrinsic Genetic Marker
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663 Protection Against Aminoglycoside-Induced Ototoxicity by AAV Vector-Mediated GDNF Gene Transfer into the Cochlea
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664 Regeneration of Sensory Cells in the Inner Ear of Deafened Mature Guinea Pigs
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665 Experiences with Delivery of Nanoparticles to the Mouse Cochlea
Mark Praxtorius, Kim Baker, Bernhard Schick, Peter K. Plinkert, Hinrich Staecker

666 LV with Different Promoters Mediated Gene Expression in the Inner Ear
Maoli Duan, Anders Fridberger, Dongguang Wei, Cecilia Lundberg

667 Lentiviral Gene Transfer of RPE65 cDNA in Knock-Out Mouse Models of Leber Congenital Amaurosis
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668 Non-Viral Gene Delivery for Ocular Diseases with Compacted DNA Nanoparticles
Rafal Farjo, Jeff Skaggs, Alexander B. Quiambao, Mark J. Cooper, Muna I. Naash

669 Sonic Hedgehog (SHH) Is Required for Retinal Angiogenesis and Its Inhibition Prevents Retinal Neovascularization
Enrico M. Surae, Alessandra Tessitore, Gabriella Cotugno, Aniello Vitale, Alberto Aurichio

670 Mutation Independent Silencing of Genes Involved in Retinal Degeneration by RNA Interference (RNAi) and Trans Complementation by a Codon Exchanged RNAi-Resistant Transgene
Siobhan Cashman, Erin Binkley, Rajendra Kumar-Singh

671 Nerve Growth Factor and Neurotrophin-3 Delivered by HSV Gene Transfer Reverses Diabetic Neuropathy

Gene Therapy for Connective Tissue

672 The Effect of Serine Protease Inhibitor 6 (SPI-6) Gene Transfer on a Mouse Model of Sjögren’s Syndrome
Fumi Mineshiba, Beatrisa M. Lodde, Jianghua Wang, Livia A. Casciola-Rosen, Antony Rosen, Bruce J. Baum

673 Plasmid-Based GHRH Supplementation Delivered by Electroproporation as a Treatment of Arthritis/Laminitis in Horses
Patricia A. Brown, David M. Hood, Ruxandra Draghia-Akli

674 Molecular, Biochemical and Biomechanical Analysis of Articular Cartilage Repaired with Genetically Modified Chondrocytes Expressing Insulin-Like Growth Factor-I

675 Enhanced Survival of Chondrocytes over Expressing Insulin like Growth Factor I in Equine Cartilage Repair
Laurie R. Goodrich, Chisa Hidaka, Mary L. Strassheim, Paul D. Robbins, Chris H. Evans, Alan J. Nixon

676 Local Stimulation of Articular Cartilage Repair by Transplantation of Encapsulated Chondrocytes Overexpressing Human FGF-2 In Vivo
Gunter Kaul, Magdi Chacchiarini, David Zurakowski, Stephen B. Trippel, Dieter Kohn, Henning Madry

677 Gene Delivery of TNFR:Fc by Adeno-Associated Virus Vector Blocks Progression of Periodontitis
Mario Taba Jr, Heather H. Huffer, Charles E. Shelburne, Jaclynn M. Kriegl, Steven A. Goldstein, Kurt H. Lustig, Haim Burstein, William V. Giannobile

678 Inflammatory Cytokines Regulate Transgene Expression from Rheumatoid Synoviocytes Infected with Double Stranded Adeno-Associated Virus Vectors
Russell S. Traister, Sylvie Fabre, Xiao Xiao, Raphael Hirsch

679 Establishment of New IL-6 Receptor Inhibitor Applicable to the Gene Therapy
Yasuo Adachi, Naoko Yoshio, Chieko Aoki, Alexander Pereboev, David T. Curiel, Masato Yamamoto, Norihiro Nishimoto

680 Application of Gene Therapy to Tendon Healing: Modification of Tenocytes with Exogenous PDGF and VEGF Genes to Promote Collagen Production
Jin Bo Tang, Xiao Tian Wang, Paul Y. Liu

681 Hydrogel Encapsulation of Adenovirus-Transduced Cells Expressing BMP2 for Local Osteoinduction
Malavosklish Bikram, Christine Fouletier-Dilling, Andre M. Gobin, Alan R. Davis, Elizabeth A. Olmsted-Davis, Jennifer L. West

682 Over-Expression of Follistatin-Like Protein Exacerbates Collagen Induced Arthritis
Takako Miyamae, Anthony D. Marinov, David C. Wilson, Raphael Hirsch

683 Adenoviral Vector-Mediated Expression of Bone Morphogenetic Protein-4 in Mesenchymal Stem Cells Induces Differentiation into the Osteoblastic Lineage and Bone Production In Vivo
Barbara Lombardo, Rosa Di Noto, Maria Teresa Esposito, Sabrina Battista, Gabriela Cipietti, Ombretta Capitani, Luigi Nicolaoli, Paolo Netti, Francesco Salvatore, Lucio Pastore

684 Viral Gene Therapy in Tendon Healing: Adeno-Associated Virus-2 but Not Other Serotypes Effectively Transduces Intrasynovial Tenocytes with Persistent Expression of the Transgene
Xiao Tian Wang, Paul Y. Liu, Jin Bo Tang
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685  Inhibitory Effect of Ribbon-type NF-kB Decoy Oligodeoxynucleotides on Osteoclast Induction and Activity
Yasuo Kamigiza, Tetsuya Tomita, Narrya Tomita, Mariana Kiomy Osako, Takuji Kizawa, Keishi Sekiguchi, Ryuichi Morishita, Hideki Yoshikawa

686  Bone Formation Inhibitors in Animal Sera
Jin Zhong Li, Ann-Shung Lieu, Gerald R. Hanksin, Gregory A. Helm

687  Tracking Transgene Product-Specific T Cells In Vivo
Lixin Wang, E. M. Lord, Roland Herzog

688  Adeno-Associated Virus (AAV)-Mediated Catalase Overexpression Protects the Extensor Digitorum Longous Muscle from Contraction-Induced Injury
Mingju Liu, Yongping Yue, Dongsheng Duan

689  Therapeutic Angiogenesis for Prefabrication of Ischemic Flaps with Multiple Growth Factor Gene Transfers: A Study in a Rat Model
Paul Y. Liu, Kan Liu, Xiao Tian Wang, Kimberly M. Rieger-Christ, Jin Bo Tang, Ian C. Summerhayes

690  Real Time Imaging of Myoblast Transplantation Using the Human Sodium Iodide Symporter (hNIS) as Reporter Gene
Manaf Bouchentouf, Basma F. Benabdallah, Joel Rousseau, Marcel Dumont, Jacques P. Tremblay

691  Ad-mda7/IL-24 Induces Systemic Anticancer Immunity In Vivo
Ryo Miyahara, Sanjeev Banerjee, Kouichiro Kawano, Clay Efferson, Naotake Tsuda, Yasuko Miyahara, Constantine G. Ioannides, Sunil Chada, Rajagopal Ramesh

692  Bone Marrow Derived Neural Stem-Like Cells Expressing IL-27 Exhibit Antitumor Activity in Intracranial Gliomas
Xiangpeng Yuan, Jinwei Hu, Takayuki Yoshimoto, Keith L. Black, John S. Yu

693  Regression of Established Subcutaneous B16 Melanoma Tumors after Intratumoral Delivery of a IL-15 Expressing Plasmid Followed by In Vivo Electroporation
Kenneth E. Ugen, Michelle Kutzler, Jeffrey Westover, Barbara Marerro, Chuanhai Cao, David B. Weiner, Richard Heller

694  Breast Cancer Specificity, Anti-Tumor Activity, and Reduced Toxicity of an Adenoviral Vector Encoding Interleukin-2 under Control of the Human Mammaglobin Promoter
Paggy Hew, Changxin Shi, Melissa Pativa, Jen Robertson, Chuyan Ying, Frank L. Graham, Christina Addison, Jack Gaudle, Mary M. Hit

695  Cooperative Effects of Adenoviral Vector-Mediated Interleukin 12 Gene Therapy and Radiotherapy in a Preclinical Model of Metastatic Prostate Cancer
Tetsuo Fujita, Terry L. Timme, Koji Naruishi, Nobuyuki Kusaka, Julie X. Zhu, Guang Yang, Alexei Golstov, Chenghui Ren, Chengzhen Ren, Maria T. Vlachaki Bin S. Teh, E. Brian Butler, Timothy C. Thompson

696  Tumor Vaccine Combined with Cytokines and Suicide Gene Therapy for Canine Spontaneous Melanoma
Liliana M. E. Finocchiaro, Marie E. Maminska, Pablo J. J. Castillo, Armando L. Karara, Gabriel L. Fiszman, María D. Riveros, Gerardo C. Glikin

697  Intratumoral Injection of Cytokine-Secreting Syngeneic/Allogeneic Fibroblasts Transfected with Tumor DNA Prolongs the Survival of Mice with an Intracerebral Tumor
Terry Lichtor, Roberta P. Glick, InSug O-Sullivan, Edward P. Cohen

698  IL-23 Transduced Dendritic Cells Produce a Potent Tumor-Specific Immunity Against Intracranial Gliomas
Jinwei Hu, Xiangpeng Yuan, John M. Ong, Christopher J. Wheeler, Maria L. Belladonna, John S. Yu, Keith L. Black

699  Evaluation of Potential Toxicity of Electroporation Mediated Deliver of a Plasmid Encoding for IL-12 in a Mouse Melanoma Model
Richard Heller, Adil Daud, Jeffrey Westover, Kathleen Merkler, Yolmari Cruz, Richard Gilbert, Loree Heller

700  Regression of Squamous Cell Carcinoma by Single Administration of IL-12 and B7.1 Genes Via Electroporation
Jiaguo Liu, Marina Torre, Shulin Li
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701 Enhanced Anti-Tumor Effect of Interferon Gene Transfer by Sustained Transgene Expression Using CpG-Reduced Plasmid DNA
Hiroki Kawano, Makiya Nishikawa, Masaru Mitsui, Yoshinobu Takakura

702 Concurrent Delivery of GM-CSF and B7-1 Using an Oncolytic Adenovirus Elicits Potent Anti-Tumor Effect on the Growth Established B16-F10 Tumor Model
Kyoung-Ju Choi, Jaesung Kim, Jing Hua Huang, Minjung Kim, Beum-Soek Suh, Hogen Kim, Byeong Chul Cho, Joohang Kim, Chae-Ok Yun

703 Combination Therapy with Bleomycin and IL-12 Gene Induces Regression of Breast Cancer
Marina N. Torrero, Shulin Li

704 Tumor Cell Surface Display of the Immunoglobulin Heavy Chain Fc by Gene Transfer as a Means to Mimic Antibody Therapy
Susanne Lang, David Riddle, Laura Sanz, Heung Chong, Jill Thompson, Richard Vile

Chen Jie, Jiang Minghong, Guo Minggao, Su Changqing, Wang Xinghua, Cui Zhenfu, Liu Xinyuan, Wu Mengchao, Qian Qijun

706 DNA Vaccine Strategy Against Chronic B-Cell Lymphoma: Anti-Idiotype CDR3 Vaccination
Monica Rinaldi, Daniela Fioretti, Sandra Iurescia, Emanuela Signori, Pasquale Pierimarchi, Giancarlo Tonon, Vito Michele Fazio

707 Transduction and Expansion of T Lymphocytes Genetically Engineered To Target the CD19 Antigen for the Treatment of CLL Using Xcyte™ Dynabeads®
Jolanta Stefanski, Renier J. Brentjens, Mark L. Bonyhadi, Michel Sadelain, Isabelle Riviere

708 Combination of Allogeneic Hematopoietic Stem Cell Transplantation and Allogeneic MHC Gene Transfer Against Solid Cancers
Kazunori Aoki, Masaki Ohashi, Miwa Kushida, Kimiko Yoshida, Masaki Mandai, Gary J. Nabel, Teruhiko Yoshida

709 Comparison of the GVHD-Inducing Potential and GCV Sensitivity of Naïve and Transduced and Selected CD34-TK-Expressing Murine T Cells after Allogeneic Bone Marrow Transplantation
Michael P. Rettig, Julie K. Ritchey, Bruno Neri, Mark L. Bonyhadi, John F. DiPersio

Cancer - Targeted Gene Therapy: Targeting of Non-Adenoviral Vectors

710 Tissue-Specific Gene Therapy by Replication-Competent Retroviral Vector for Prostate Cancer with Fludarabine Phosphate
Choichiro Ozu, Eiji Kikuchi, Silvia Menendez, Carlos C. Cardo, Christopher R. Logg, Noriaki Kasahara, Bernard H. Bochner

711 Treatment of Acute Myeloid Leukemia by rAAV 8 Vector Mediated Human Interferon-bd Gene Transfer
Reuben Benjamin, Andrew M. Davidoff, Pizzez Arnold, Nadir Singh, Asim Khwaja, Jenny McIntosh, Cathy C. Y. Ng, Tony Meager, Meenu Wadhwu, Amit C. Nathwani

712 Treatment of Human and Rat Hepatocellular Carcinomas by Long Term Expression of rAAV-Mediated Transfer of Human TERTC27 Polypeptide
Marie C. Lin, Samuel S. Ng, Yi Gao, ChingTung Lum, ZhenFan Yang, JunJian Huang, George K. Lau, ShuengFai Fan, Hsiang-fu Kung

713 Growth Suppression of Hepatocellular Carcinoma by AAV-Mediated Angiogenic Inhibitors
Lai-Yin Te, Ruian Xu

714 Adenoassociate Virus Mediated Long Term Expression of Human TERTC27 Polypeptide Inhibited Glioblastoma Carcinogenesis by Multiple Mechanisms
Samuel S. Ng, Yi Gao, JunJian Huang, Gui-fen Huang, Hsiao-fu Kung, Marie C. Lin

715 Noninvasive Bioluminescence Imaging of Gene Expression Following Intracerebral Gene Delivery of AAV and Adenovirus into the Mouse Brain
Perry Liu, Hideo Yuki, Heather Gibson, Harald Petry, Linda Cashion, Terry Hermiston, Katalin Kauser, Rick Harkins, Garin M. Rubanyi, Ron Vergona Hu Sheng Qian

716 Transducing Efficiency Screening for rAAV2, rAAV1, and rAAV5 in 56 Human Tumor Cell Lines
Lina Li, Linda Yang, Dominick A. Scudiero, Robert H. Shoemaker, Robert M. Kotin
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717 Cancer Gene Therapeutic Approaches Using an AAV-Mediated Gene Delivery System Containing Antisense VEGF-A cDNA
Keerang Park, Mee-Young Ahn, Seok-Yeon Huang, Yong-Hua Cho, Bong-Su Kang, Youn-Ju Kim, Sung-Ha Cho, Young-Min Kim, So-Yong Yi, Sun-Kyung Kim Eun-Jung Park, Kang-Eun Lee

718 Development of AAV and Adenovirus Cocktail Vector System for Efficient Cancer Gene Therapy by Human TERTC27 Polypeptide
Yi Gao, Samuel S. Ng, Chen Yang, David Chau, JunJian Huang, Cui-fen Huang, Hsiang-fu Kung, Marie C. Lin

719 Neutralization of Free but Not Cell Associated Oncolytic Measles Viruses by Antibody and Complement
Ianko Iankov, Stephen J. Russell

720 EGFRvIII Retargeted Oncolytic Measles Virus Strains Have Significant Antitumor Activity Against Gliomas
Cory Allen, Sompong Vongpunsawad, Takafumi Nakamura, C. David James, Mark Schroeder, Roberto Cattaneo, Stephen J. Russell, Evanthia Galanis

721 Measles Virus for Cancer Gene Therapy of Primary Liver Tumors
Boris R. A. Blechacz, Patrick Splinter, Greiner Suzanne, Mark J. Federspiel, Kah-Whye Peng, Nicholas F. LaRusso, Stephen J. Russell

722 Antitumor Effect of IL-24 Mediated by Tumor Targeting Vector ZD55 Is Much Better Than That Mediated by Replication Deficient Adenovirus
Li-h Zhao, Jin-fa Gu, Ai-wen Dong, Yan-hong Zhang, Ling-feng He, Jin-he Zhang, Yi-gang Wang, Zi-lai Zhang, Qi-jun Qian, Cheng Qian Xin-yuan Liu

723 VSV-G Pseudotyped, Semi-Replication Competent MLV Retroviruses for Cancer Gene Therapy
Jian Qiao, Jesus Moreno, Timothy Kottke, Michael Forshaw, Rosa Maria Diaz, Richard Vile

724 Herpes Simplex Virus 1 Amplicon Vector-Mediated siRNA Targeting Epidermal Growth Factor Receptor Inhibits Growth of Human Glioma Cells in Culture and In Vivo
Okay Saydam, Daniel Glauser, Irna Heid, Gulen Tirkeri, Andreas Jacobs, Mathias Ackermann, Cornel Fraefel

725 Seneca Valley Virus, a Novel Systemically Deliverable Oncolytic Virus for the Treatment of Small Cell Lung Cancer and Other Neuroendocrine Cancers
Paul L. Hallenbeck, Seshidhar P. Reddy, Shanthi Ganesh

726 Effect of G-CSF-Mediated Mobilization on Tumor Homing of Genetically Modified, Bone Marrow Derived Cells
Zong-Yi Li, Shaoheng Ni, Hans-Peter Kiem, Andre Lieber

727 Gene-Modified Mesenchymal Stem Cells Selectively Engraft in Stroma of Ovarian Carcinomas and Control Tumor Growth
Jennifer Dembinski, Matus Studeny, Claudia Zompetta, Michael Andreuff, Frank Marini

728 Anti-TAG-72 Immunoliposomes for Efficient Tumor-Targeted Gene Therapy
Keun-Sik Kim, Hong Sung Kim, Eun Jung Kim, Jong Chul Kim, In-Ho Song, Hye Jeong Hong, Yong Seok Park

729 C-Met Antisense Plasmid Liposome Complex Increases Radiation Sensitivity of Lung Tumor Cells In Vivo or In Vitro
Yunyun Niu, Laura Stabile, Michael W. Epperly, Jill Siegfried, Joel S. Greenberger

730 Cancer Specific Promoters for Gene Therapy of Small Cell Lung Cancer
Nina Pedersen, Mikkel W. Pedersen, Michael S. Lan, Thomas T. Poulsen, Hans S. Poulsen

731 Completely Elimination of Xenograft SW620 Cancer by Combined Tumor Targeting MnSOD and Trail Genes
Yan-hong Zhang, Jin-fa Gu, Li-li Zhao, Yi-gang Wang, Jin-hui Wang, Ling-feng He, Wei-guo Zou, Qi-Jun Qian, Cheng Qian, Xin-yan Liu

732 Defining Tropism of Oncolytic Vectors by Protease Availability: Measles Viruses Selectively Fusing Matrix-Metalloproteinase Expressing Cells
Christoph Springfeld, Veronika von Messling, Christian Buchholz, Roberto Cattaneo

Gene Regulation: Overcoming Obstacles to Gene Transfer and Expression

733 The Human XIST Gene Promoter Prevents Silencing of an Integrated Reporter Gene
Michael R. Greene, Christopher H. Lourey
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FRIDAY, JUNE 3

734
Association of DNA Polymerase μ with Recombinant Adeno-Associated Viral Vectors
David C. Brooks, Fayaz R. Khazi, Katherine A. High

735
Femtosecond Laser- A New Intradermal DNA Delivery Method for Gene Expression and Vaccination
Evelyne Zeira, Alexandre Manervitch, Zakaria Manervitch, Menahem Harati, Hagit Yotvat, Michal Gropp, Orit Papo, Nili Daudi, Eli Kedar, Aaron Lewis Eithan Galun

736
Novel In Vitro and In Vivo Transcription Pattern Analysis Identifies New Adenovirus Responsive, Innate Gene Networks
Zachary Hartman, Anne Kiang, Ruth Everett, Joe Nevins, Esther Black, Andy Amalfiutano

737
Induction of Molecular Chimerism in Rhesus Macaques Reconstituted with Autologous CD34+ Cells Transduced with the Gene Encoding the α(1-3)galactosyltransferase (αGT)
Lorenzo Benatuil, Robert Donahue, Aylin Bonifacino, Cynthia Dunbar, John Iacomini

738
Influence of the Major Histocompatibility Complex on Immunological Rejection of Skin Grafts Expressing a Gene Therapy Reporter Gene
Julian D. Down, Christian LeGuern

739
Impact of Activation, Transduction/Selection Steps on the Gene Expression Profile of Ex-Vivo Retrovirally Transduced CD8+ T Cells, Using High Density DNA Microarrays
Marina Deschamps, Jean Marie Certoux, John de Vos, Nicolas Montcuquet, Thierry Rene, Mark Bonyhadi, Eric Robinet, Pierre Tiberghien, Christophe Ferrand

740
In Vitro Correction of 47 bp Deletion in Casp-3 Gene in Human MCF7 Cells Using Histone-Mediated Delivery of Wild Type Gene Fragment Covering the Deletion Region
Leonid A. Yakubov, Robert J. Levy

741
AAV Delivered Ribozymes to Recapitulate the Early Stages of Age Related Macular Degeneration
Verline Justilien, Jijing Pang, William W. Hauswirth, Alfred S. Lewin

742
Human Artificial Chromosome (HAC) Vector Provides Long-Term Therapeutic Transgene Expression in Normal Human Primary Fibroblasts
Minoru Kakeda, Masaharu Hiratsuka, Keiko Nagata, Akiko Sano, Kanako Osawa, Motonobu Katoh, Mitsu Oshimura, Kazuma Tomizuka

743
Lentiviral Vector Gene Transfer in Monkeys: In Vivo Detection of Gene Expression Longitudinally Using MicroPET and Optical Imaging
Alice F. Tarantal, Chang I. Lee, Daniel F. Jimenez, Sanjiv S. Gambhir, Donald B. Kohn, John J. Rossi, Simon R. Cherry

744
Enhanced Expression of Adenovirus Transgenes in the Mouse Liver and Human Tumors Following Topoisomerase I Inhibition
Gideon Zamir, Evelyne Zeira, Eithan Galun

745
CD4-T Cell Responses Mediated by IFNγ and Perforin Eliminate Adenoviral-Mediated Transgene Expression from the CNS of Mice by Cytolytic and Non-Cytolytic Mechanisms
Jeffrey M. Zieger, Carlos Barcia, Chunyan Liu, Maria Castro, Pedro Lowenstein

746
Evaluation of Nuclear Transfer and Transcription of Plasmid DNA Condensed with Protamine
Tomoya Masuda, Hidetaka Akita, Hideyoshi Harashima

747
Identification and Characterization of Mutations within the Coxsackievirus and Adenovirus Receptor (CAR)
Katherine J. D. A. Excoffon, Matthew Avenarius, Richard Smith, Joseph Zabner

748
Quantitative Comparison of the Intracellular Trafficking and Expression Pattern of the Exogenous Genes between the Adenovirus and Artificial Vector
Susumu Hama, Hidetaka Akita, Rie Ito, Hiroyuki Mizuguchi, Taka Hayakawa, Hideyoshi Harashima

749
Characterization of Adenovirus-Mediated Gene Expression in Normal, Wounded and Wound Impaired Skin
Danling Gu, David Looper, David W. Kang, Diane McAllister, Erlinda Quijano, Shu Fen Wen, Monica L. Zepeda

750
Expression Levels of the Amphotropic Receptor May Explain Gestational Age-Dependence of Tissue Transduction in Fetal Sheep
Ferhat Ozturk, Paul Park, Joe Tellez, Wayne B. Anderson, Maribeth Eiden, Esmail D. Zanjani, Graca Almeida-Porada, Christopher D. Porada
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FRIDAY, JUNE 3

751 High Efficient Ex Vivo Gene Delivery into Human Corneal Endothelial Cells by Recombinant Adeno-Associated Virus
Li-Ju Lai, Juan Li, Samuel Chao-Ming Huang, Ken-Kuo Lin, Xiao Xiao

752 Equine Infectious Anemia Virus Pseudotyped with the Vesicular Stomatitis Virus G-Protein, Preferentially Targets Neural Precursors in the Adult Mouse Brain
Troy Ghashghaei, Manij Patel, John Olsen, Eva Anton

753 Efficient Gene Therapy by Intercellular Spread and Specific Internalization of the Transgene Product
Antoine M. Beerens, Marianne G. Rots, Hidde J. Haisma

754 Different Regulation of Myocardial Atrial and Ventricular Calcium Pump Activity (Ca-ATPase) during Cardiac Arrest

SATURDAY, JUNE 4

Oral Abstract Session 410 DNA Viruses: Vaccines, HSV, Baculovirus and SV40 Room: 240 Complex

755 10:15 AM Vaccinia Deleted Multiple Anti-Apoptosis Viral Genes Enhances Its Specificity as an Oncolytic Virus
Shuting Yang, Zongheng Guo, Mark E. O’Malley, David L. Bartlett

756 10:30 AM The Use of Immune Cell Trafficking to Deliver an Oncolytic Virus to Tumors
Steve H. Thorne, Robert Negrin, Christopher H. Contag

757 10:45 AM Replication Defective HSV-1 Immediate Early (IE) Gene Mutants: A Comparison of Vector Toxicity Versus Transgene Expression
David M. Krisky, James Wechuck, Darren Wolfe, Shaohua Huang, Ali Ozuer, Joseph C. Glorioso

758 11:00 AM A Multipurpose Vector System for the Simultaneous Screening of Libraries in Bacteria, Insect and Mammalian Cells and Expression In Vivo

759 11:15 AM Liver Transduction with an SV40 Vector Encoding Insulin-Like Growth Factor I Reduces Hepatic Damage and Delays Cirrhosis Progression
Maria Vera, Laura Martinez, Mikel Zaratiegui, Carlos Rodriguez, Jesus Prieto, Puri Fortes

760 11:30 AM Development of an HSV-Based Model System to Identify Factors Acting in Embryonic Myogenesis
April M. Sunyog, Darren P. Wolfe, David Krisky, James Wechuck, Ying Jiang, Joseph C. Glorioso

761 11:45 AM Dominant-Negative FGF Receptor Expression by Oncolytic HSV Vector for the Treatment of Malignant Peripheral Nerve Sheath Tumors
Tä-Chiang Liu, Tingguo Zhang, Hiroshi Fukuhara, Toshi Kuroda, Robert L. Martuza, Andreas Kurtz, Samuel D. Rabkin
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SATURDAY, JUNE 4

762  12:00 PM
Cisplatin-Induced DNA Damage Repair Mechanism Potentiates the Synergistic Efficacy of Oncolytic Herpes Simplex Viral Therapy in the Treatment of Malignant Mesothelioma
Prasad S. Adusumilli, Mei-Ki Chan, David P. Eisenberg, Zhenkun Yu, Karen Hendershott, Ting-Chao Chou, Valerie W. Rusch, Yuman Fong

Oral Abstract Session 411
Naked DNA Gene Transfer: Optimizing Expression/Animal Models
Room: 260/264

763  10:15 AM
Optimization of Plasmid Components for Gene Therapeutic Purposes
Ruxandra Draghia-Akli, Melissa A. Pope, Amir S. Khan

764  10:30 AM
RNA and Codon Optimized HIV Candidate Vaccines – from Bench to Clinical Trials
Ralf Wagner, Petra Mooij, Sunita Balla, Jens Wild, Kurt Bieler, Hans Wolf, Frank Nutka, Jonathan Heeney, Giuseppe Pun taleo

765  10:45 AM
Intravascular Delivery of Naked Plasmid DNA into Muscle for Long-Term Correction of Hyperbilirubinemia in the Gunn Rat
Istvan Danko, Zhen Jia

766  11:00 AM
Bone Regeneration Induced by Combining In Vivo Electroporation of an Osteogenic Gene and Human Mesenchymal Stem Cells
Gadi Pelled, A Lazarus, Yoram Zilberman, Eiveline Zeira, Hagit Yonat, Ethan Galun, Jin Zhong Li, Gregory A. Helm, Dan Gazit

767  11:15 AM
Role of the Cytoskeleton in Intracellular Plasmid Movement
Erin E. Vaughan, David A. Dean

768  11:30 AM
Two Distinct Mechanisms, Silencing of RNA Expression and Loss of Vector DNA, Are Responsible for the Loss of Transgene Expression Following Delivery of Foreign Genes to Skeletal Muscle
Alan R. Brooks, Peijin Wang, Paul Szymanski, Perry Liu, Heather Gibson, Hu Sheng Qian, Rick N. Harkins, Gabor M. Rubanyi

769  11:45 AM
Tissue Engineering Scaffolds with Non-Viral Vectors: Sustained and Substrate-Mediated Delivery
Jae-Hyung Jang, Christopher B. Rives, Zain Bengali, Lonnie D. Shea

770  12:00 PM
Novel Physical Methods for Nonviral DNA Transfer to Rabbit Retina
Thomas W. Chalberg, Alexander Vanov, Philip Huie, Fumiki E. Mohan, Michele P. Calos, Daniel V. Almanker

Oral Abstract Session 412
Lipid Mediated Gene Transfer: Non-Viral Delivery Systems
Room: 263/267

771  10:15 AM
Complexes of Nucleic Acids with Multivalent Cationic Lipids for the Study of Delivery Pathways and Mechanisms: From Plasmid DNA to siRNA
Kai Ewert, Nathan F. Bouxsein, Christopher S. McAllister, Heather M. Evans, Charles E. Samuel, Cyrus R. Saffi

772  10:30 AM
Convective Flow Increases Lipoplex Delivery Rate to In Vitro Cellular Monolayers
Steven S. Harris, Todd D. Giorgio

773  10:45 AM
Role of Lipid-Rafts in the Binding and Internalization of Dendriplexes and Lipoplexes
Peng H. Tan, Maria Mamunta, Andrew J. T. George

774  11:00 AM
Studies on the Intracellular Release of Genetic Drugs from Pharmaceutical Carriers
Bart Lucas, Katrien Remaut, Stefaan C. De Smedt, Roosmarijn Vandenbroucke, Joseph Demeester

775  11:15 AM
Substrate-Mediated DNA Delivery for Neural Tissue Engineering
Tiffany L. Houchin, Jae-Hyung Jang, Lonnie D. Shea

776  11:30 AM
A Novel Dual Functioning Non-Viral Vector - Delivering Genes While Targeting Nuclear Receptor Signaling
Peng Liu, Christine Connell, Lisa Stolz, M. Scollenberger, Leaf Huang

777  11:45 AM
Polymer Library Approaches to Gene Therapy
Daniel G. Anderson, Andreas Zumbuehl, Weidun Peng, Janet A. Sawicki, Robert Langer

778  12:00 PM
Nocodazole Enhances CMV Promoter Activity in a Human Pancreatic Cancer (Panc 1) Cell through Activation of c-Jun
Robert Arpke, Pi-Wan Cheng
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SATURDAY, JUNE 4

Oral Abstract Session 413
Molecular Conjugates: Therapeutic Applications
Room: 120/124

779 10:15 AM
Oral Delivery of Non-Viral DNA Nanoparticles for Hemophilia A
Katherine Bowman, Riita Sarkar, Sanj Raut, Neelima Shah, Xuli Wang, Hai-Quan Mao, Kam W. Leong

780 10:30 AM
A Novel Multi-Component Molecular Conjugate Vector for Highly Efficient p53-Mediated Tumor-Specific Gene Therapy
Stanley Saamoah-Moffatt, Sandra Wiehle, Richard J. Cristiano

781 10:45 AM
The Effect of Overexpression of Lysyl Oxidase on Dermal Wound Healing
Ying-Ka I. Lau, Jennifer L. West

782 11:00 AM
Poly(glycoamidoamine)s Deliver DNA with High Efficacy to Cardiomyoblast Cells
Yemin Liu, Suwen He, W. Keith Jones, Theresa M. Reineke

783 11:15 AM
Transient Transduction of Repopulating Human CD34+ Cells Using Nanoparticles in a Clinically Applicable Ex Vivo Protocol
Jesper Bonde, Dustin J. Maxwell, David A. Hess, Ryan Lahey, David Piwnica-Worms, Jan A. Nolta

784 11:30 AM
Promoter-Dependence of Plasmid-Pluronics Targeted alpha-Galactosidase A Expression in Skeletal Muscle of Fabry Mice
Matthieu D. Lavigne, Marita Pohlschmidt, Javier F. Novo, Valery Alakhov, Dariusz C. Gorecki

785 11:45 AM
Cell-Permeable Peptides Mediated Gene Delivery in Hepatocytes In Vitro and In Vivo
Zhenping Shen, Carol H. Miao

786 12:00 PM
Synthesis of Cationic Biodegradable Polymers for Targeted Gene Delivery to Hepatocytes
Gregory T. Zugates, Daniel Anderson, Ingrid Lawhorn, Robert Langer

Oral Abstract Session 414
Muscle and Connective Tissue: Gene Therapy for Muscular Dystrophy
Room: 230/231

787 10:15 AM
Lentivirus Mediated Dystrophin Expression in mdx Muscles
En Kimura, Sheng Li, Brent Fall, Miki Haraguchi, Leonard Meuse, Jeffery S. Chamberlain

788 10:30 AM
Antisense Therapy for Duchenne Muscular Dystrophy: A Realistic Possibility
Qi L. Lu, Adam Rubinowitz, HaiFang Yin, Julia Alter, George Bou-Gharios, Terrence Partridge

789 10:45 AM
Stable Genome Alteration of the Dystrophin Gene for the Treatment of Duchenne Muscular Dystrophy (DMD) Due to Frame-Shift Mutations Using Oligonucleotide-Mediated Gene Editing
Carmen Bertoni, Thomas A. Rando

790 11:00 AM
Evaluation of Immune Responses to Dystrophin in Humanized mdx HLA-A*0201 Dystrophic Mice: Application in Duchenne Muscular Dystrophy Gene Therapy after U7 snRNA-Mediated Exon Skipping
Florent Ginhoux, Sabrina Turbant, Maryline Leboeuf, David A. Gross, Francois Lemonnier, Luis Garcia, Olivier Danos, Jean Davoust

791 11:15 AM
Immunity to AAV-Mediated Gene Therapy in a Random-Bred Canine Model of Duchenne Muscular Dystrophy
Zejing Wang, Michael J. Blankinship, Paul Gregorevic, Marie-Terese Little, Rainier J. Storb, James M. Allen, Stephen J. Tapscott, Jeffrey S. Chamberlain, Christian S. Kuhr

792 11:30 AM
Canine Mini-Dystrophin Gene Transfer by AAV1 in mdx Mice Ameliorates Dystrophic Pathology and Protects Membrane Integrity
Bing Wang, Juan Li, Libiao Zhou, Chunping Qiao, Mengnan Tian, Tong Zhu, Janet Bogan, Joseph Korngay, Xiao Xiao

793 11:45 AM
Efficient Expression of the 6 kb Mini-Dystrophin Gene by Trans-Splicing Adeno-Associated Viral (AAV) Vector Restores the Entire Dystrophin-Associated Glycoprotein Complex and Reduces Contraction-Induced Damage in the Mdx Mouse Model of Duchenne Muscular Dystrophy
Yi Lai, Yongping Yue, Mingju Liu, Arka Ghosh, John F. Engelhardt, Jeffrey S. Chamberlain, Dongsheng Duan
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794 12:00 PM
Evaluation of Gene Transfer Efficacy Mediated by AAV1 and AAV6 Vectors in Skeletal Muscle of Adult Mice Following Different Routes of Administration
Christel Rivière, Karine Poulard, Gabor Véres, Olivier Danos, Anne M. Douar
Oral Abstract Session 415
Cellular Immunotherapy of Cancer Using Genetically Modified Effectors
Room: Ferrara Theater

795 10:15 AM
The Use of Autologous LMP2-Specific Cytotoxic T Lymphocytes for the Treatment of Relapsed EBV +ve Hodgkin Disease and Non-Hodgkin Lymphoma
Catherine M. Bollard, Stephen Gottschalk, Elizabeth Baza, Helen Huls, Vicky Gresik, George Carrum, Malcolm K. Brenner, Cliona M. Rooney, Helen E Heslop
796 10:30 AM
In Vivo Elimination of Human Leukaemia Cells by WT1 Specific TCR Transduced T Cells
Shao-An Xue, Liqian Gao, Daniel Hart, Waseem Qasim, Adrian Thrasher, Jane Apperley, Wolfgang Uckert, Emma Morris, Hans Stauss
797 10:45 AM
Targeted Elimination of Prostate Cancer by Genetically Directed Human T Lymphocytes
Matthias Stephan, Terence F. F. Gade, Wádiel Hassen, Elmer Santos, Gertrude Gunset
798 11:00 AM
Epstein Barr Virus-Specific Cytotoxic T Lymphocytes (EBV-CTL) Expressing an Anti-CD30 Chimeric T-Cell Receptor (cTcR) for the Treatment of Hodgkin’s Disease (HD)
Barbara Savoldo, Cliona Rooney, Helen Heslop, Hinrich Abken, Andreas Hombach, Lan Zhang, Martin Pule, Gianpietro Dotti, Malcolm Brenner
799 11:15 AM
In Vitro and In Vivo Characterization of “Second-Generation” Co-Stimulatory Chimeric Antigen Receptors (CARs) Targeting the CD19 Antigen Present on B Cell Malignancies
Renier J. Brentjens, Yan Nikhamin, Miko Matsushita, Michel Sadelaar
800 11:30 AM
Adenoviral Transduced Monocytes: Efficient Antigen Presenting Cells for the Activation of Antigen-Specific T Cells
Ann M. Leen, Maheshika K. Ratnayake, Aaron E. Foster, Cliona M. Rooney, Stephen Gottschalk

801 11:45 AM
Fas Knockdown Mediated by siRNA in EBV-Specific Cytotoxic T-Lymphocytes (CTL) Reduces Their Sensitivity to Fas/FasL-Induced Apoptosis
Gianpietro Dotti, Barbara Savoldo, Martin Pule, Karin C. Straathof, Ettore Biagi, Eric Yvon, Stéphane Vigouroux, Cliona M. Rooney, Malcolm K. Brenner
802 12:00 PM
Use of Chemokine Expression at a Tumor Site to Enhance Immune Cell Trafficking for Melanoma Therapy
Uma Thanarajasingam, Laura Sanz, Rosa Maria Diaz, Jill Thompson, Michael Gough, Richard Vile

803 10:15 AM
The Effects of Intratumoral Injection of a Replicating Morbillivirus in a Canine Model of Naturally Occurring Lymphoma
Michael S. Henson, Steven E. Suter, Veronika A. von Messling, Roberto Cattaneo, Adele K. Fielding
804 10:45 AM
HSVtk Mediated Immunostimulatory Tumor Cell Killing Enhances Adoptive T Cell Therapy in a B16 Melanoma Model
Luis Sanchez-Perez, Michael Gough, Jian Qiao, Uma Thanarajasingam, Atique Ahmed, Rosa Maria Diaz, Richard Vile
805 10:45 AM
Effective Suicide Gene Therapy of Leukemia in a Novel Model of Retroviral Insertion
Martina Blumenthal, Dianne Skelton, Denise A. Carbonaro, Karen A. Pepper, Donald B. Kohn
806 11:00 AM
Phase I/II Trial of Intravenous NDV-HUJ (OV001) Oncolytic Virus in Recurrent Glioblastoma Multiforme
Arnold I. Freeman, Zichria Zakay-Rones, John M. Gomori, Eduard Linetsky, Linda Rasooly, Amos Panet, Eugene Libson, Charles S. Irving, Tali Siegal, Ethan Galun
807 11:15 AM
Addition of HU to GCV Induces Bystander Cytotoxicity in Co-Cultures of HSVTK-Expressing and Non-Expressing HeLa Cells
Brian G. Gentry, Paul D. Boucher, Donna S. Shewach
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808 11:30 AM
Monitoring of Transgene Expression in Cancer Patients with Positron Emission Tomography (PET)
Bruno Sangro, Ivan Penuelas, Guillermo Mazzolini, Jose J. Boam, Josep M. Marti-Climent, Maria Ruiz, Cheng Qian, Nagichettiar Satyamurthy, Jorge R. Barrio, Michael E. Phelps
Jose A. Richter, Sam Gambhir, Jesus Prieto

809 11:45 AM
mda-7 Kills Pancreatic Cancer Cells by Inhibition of Wnt/PI3K Signaling: Identification of IL-20 Receptor-Mediated Bystander Activity Against Pancreatic Cancer
Suril Chada, Dora Bocangel, Ramesh Rajagopal, Elizabeth Grimm, Abner M. Mhashilkar, Mingzhong Zheng

810 12:00 PM
Ex-Vivo Activation and Genetic Manipulation of Human T Cells Results in Consistent In-Vivo Expansion and Lethal GvHD in a Novel Murine Xenotransplant Model
Bruno Nervi, Michael P. Rettig, Julie K. Ritchey, Gerhard Bauer, Jon Walker, Phillip E. Herrbrich, Todd E. Meyerrose, Mark Bonyhadi, Jan A. Nolta, John F. DiPersio

Oral Abstract Session 417
Gene Therapy Approaches to Pulmonary Disease
Room: 261/262

811 10:15 AM
ACE-Targeted eNOS and BMPR2 Gene Therapy Attenuates Pulmonary Hypertension in a Chronic Hypoxia Rat Model
Ann M. Reynolds, Mark D. Holmes, David T. Curiel, Andrew H. Baker, Paul N. Reynolds

812 10:30 AM
Non-Human Primate Serotype rh.10 AAV Directed Therapeutic Serum Levels of α1-antitrypsin Following Intrapleural Administration in Mice with Pre-Existing Immunity to AAV
Bishnu P. De, Adriana Heguy, Neil R. Hackett, John Lee, Guangping Gao, James M. Wilson, Ronald G. Crystal

813 10:45 AM
Distribution of Transgene Expressing Cells Following Intramarrow Injection to Transduce Hematopoietic Stem Cells In Situ Using rSV40 Vectors
Jean-Pierre Louboutin, Biaoning Liu, David S. Strayer

814 11:00 AM
Adenovirus-Mediated Gene Transfer Demonstrates That Pirin, a Transcription Factor Up-Regulated in the Bronchial Epithelium by Cigarette Smoke, Mediates Bronchial Epithelial Cell Apoptosis
Brian D. Gelbman, Adriana Heguy, Timothy P. O'Connor, Joseph Zabner, Ronald G. Crystal

815 11:15 AM
Aerosol Delivery of Helper-Dependent Adenoviral Vector into Nonhuman Primate Lungs Results in High Efficiency Pulmonary Transduction with Minimal Toxicity
Peter Hiatt, Nicola Brunetti-Pierri, David Koehler, Ruth McConnell, Julie Katkin, Donna Palmer, David Dimmock, Jim Hu, Milton Finngold, Arthur Beaudet Dee Carey, Karen Rice, Philip Ng

816 11:30 AM
RNAi Applications for Respiratory Epithelia: Delivery and Efficacy
Anthony J. Fischer, Christine L. Wohlford-Lenane, Patrick L. Sinn, Hong Peng Jia, Steven M. Varga, Beverly L. Davidson, Paul B. McMat

817 11:45 AM
Protein Transduction Domain Mediated DFRD Correction of CFTR Function in Airway Cells from DF508 Homozygous CF Patients
Zhibao Mi, Fei Sun, Xiaoli Lu, Steve Condliffe, Xiaoyan Gong, Joseph Pilewski, Raymond A. Frizzell, Paul D. Robbins

818 12:00 PM
Recombinant AAV-Mediated Treatment of CNS Glycogen Accumulation in Mouse Model of Glycogen Storage Disease Type II
Lara R. DeRuisseau, Cathryn S. Mah, Thomas J. Faites, Jr., David D. Fuller, Barry J. Byrne

Presidential Symposium 430
Room: America’s Ballroom

819 3:20 PM
Molecular Follow-Up of Patients Treated with Allogeneic Hematopoietic Stem Cell Transplantation and Donor Lymphocytes Transduced with a Retroviral Vector Expressing HSV-TK and ΔLNGFR
Alessandra Recchia, Zulma Magnani, Fabrizia Urbinati, Daniela Sartori, Sara Muraro, Maria T. Lupi Stanghellini, Massimo Bernardi, Alessandra Pescarollo, Marco Brogni, Chiara Bonini Claudio Bordignon, Fabio Ciceri, Fulvio Mavilio

820 3:38 PM
Highly Efficient Exon-Skipping and Sustained Correction of Muscular Dystrophy Using an Adeno-Associated Viral Vector
Audrie Goyenvalle, Adeline Valin, Jean Claude Kaplan, France Leturcq, Olivier Donos, Luis Garcia

821 3:56 PM
Development of a Site-Specific Integrating Gene-Deleted Adenoviral Vector for Sustained Transgene Expression Levels In Vivo
Anja Ehrhardt, Richard M. Yant, Giering A. Jeff, Hui Xu, Michele P. Calos, Mark A. Kay
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Poster Session III
Exhibit Hall 2

RNA Virus Vectors: Safety, Therapeutics
(Note: Abstract 822 has been moved from Poster Session III to Poster Session I and will follow Abstract 109.)

822
A Rev/RRE Dependent Packaging System for MLV Based Vectors Raises Biosafety Concerns
Hong Ma, Adam Cockrell, Ryan Bash, Terry VanDyke, Tal Kafri

823
Dissecting Overlapping Functions of Transcriptional Enhancer and Polyadenylation in the Lentiviral U3
Qing Yang, Wayne Chou, Lung-Ji Chang

824
Oncoretroviral Vector Integration Biases in Mouse Hematopoietic Progenitors
Mari Aker, Julie Tubb, George Stamatoyannopoulos, David W. Emery

825
Product Enhanced Reverse Transcriptase (PERT) Assay for RCL/RCR Detection
Lakshmi Sastry, Yi Xu, Lisa Duffy, Sue Koop, Aparna Jasti, Holger Roehl, Doug Jolly, Kenneth Cornetta

826
The Risk of Integration: Characterization and Testing of Cell Lines Engineered for the Study of Insertional Gene Activation
R. Scott McIvor, Nikunj V. Somia, Andrea D. Converse

827
Integration and Biodistribution Studies of Retroviral Vector in Mouse Gonad Tissues
Gyu Seek Rhee, Ji hyun Seok, Soon Sun Kim, Seung Jun Kwack, Bo Ra Kim, Rhee Da Lee, Soo Yong Chae, Young Hyuk Won, Seung Shin Yu, Dae Hyun Cho

828
Optimizing the Woodchuck Hepatitis Virus Post-Transcriptional Regulatory Element (WPRE) for Safety and Function in Lentiviral Vectors
Manij Patel, John C. Olsen

829
Design of a Safe and Efficient Vector for Bone Marrow Chemoprotection by O6-Methyl-Guanine-DNA-MethylTransferase
Axel Schambach, Jens Bohné, Saurabh Chandra, Elke Will, Lars Mueller, Geoffrey Margison, David A. Williams, Christopher Baum

830
Determination of the Profile and Site Preference of Lentiviral Vector Integration
Caroline V. Hacker, Susan M. Kingsman, Kyriacos A. Mitrophanous, James E. Miskin

831
Evaluation of Lentiviral Vectors for Gene Therapy of Wiskott-Aldrich Syndrome
Marguerite V. Evans-Galea, Matthew M. Wielgosz, Hideki Hanawa, Arthur W. Nienhuis

832
Suppression of HIV-1 Replication by Delivery of multiple siRNA Genes Against p24, Tat/Rev and CCR5
Hsin-Lung Lo, Jerry Wu, Tan Chang, Priscilla Yam, John Zaia, Jiing-Kuan Yee

833
A Preclinical Safety Study of Simian Immunodeficiency Virus (SIV)-Based Lentivirus Vector for Retinal Gene Transfer in Non-Human Primates
Yasuhiro Ikeda, Yoshikazu Yonemitsu, Masanori Miyazaki, Riichiro Kono, Toshinori Murata, Yoshinobu Goto, Toshiaki Tabata, Yasuyi Ueda, Naohide Aoyama, Keiji Terao Mamoru Hasogawa, Tatsuro Ishibashi, Katsuo Sueishi

834
Highly Efficient and Tumor-Selective Gene Delivery in Metastatic Colon Cancer Models Using a Replication-Competent Retrovirus Vector
Kei Hiraoka, Takahiro Kimura, Christopher Logg, Satoshi Kondo, Noriyuki Kasahara

835
Gene Therapy for Arthritis: Long-Term Expression in a Sub-Population of Synovial Fibroblasts
Elvire Gouze, Jean-Noel Gouze, Glyn D. Palmer, Carmencita Pilapil, Christopher H. Evans, Steven C. Ghivizzani

836
Suitability of Anti-Retroviral Genes for Gene Therapy of AIDS
Matthias Schweizer, Sabine Fengler, Silke Schuele, Sylvia Raupp, Carsten Muenk, Dorothee M. von Laer, Klaus Cichutek

837
Prolonged Gene Expression in Murine Salivary Glands Following FIV-Based Lentiviral Vector Transduction
Ela Shai, Reba Condiotti, Michael A. Curran, Garry P. Nolan, Ethan Galun

838
Construction and Use of Retroviral Vectors Encoding the Toxic Gene Barnase
Sumit Agarwal, Bryan Nikolai, Tomoyuki Yamaguchi, Nikunj V. Somia
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839
Lentiviral Vectors Encoding Connexin43 Mutants Reduce Gap Junction Function: Toward Molecular Ablation of Re-Entrant Cardiac Arrhythmias
Eddy Kizana, Samantha L. Ginn, Christine Smyth, Anita Boyd, Stuart P. Thomas, David G. Allen, David L. Ross, Ian E. Alexander

840
Recombinant Sendai Virus Vectors with Matrix Protein Gene Deletion Restore Dendritic Cell Allostimulatory Activity
Sorin Armeanu, Irina Smirnow, Sascha Bossou, Wolfgang J. Neubert, Ulrich M. Lauer, Peter Brossart, Michael Bitzer

841
A Cytoplasmic Gene Therapy with Sendai Virus Vectors: Reduction of Cytopathic and Immunogenic Reaction of New Class Cytoplasmic RNA Vector
Makoto Inoue, Hitoshi Iwasaki, Mariko Yoshizaki, Akihiro Tagawa, Kentaro Washizawa, Takashi Hironaka, Mamoru Hasegawa

842
Repeated Administration of Sendai Virus into Lung
Uta Griesenbach, Gerry McLachlan, Luci Somerton, David Collie, Makoto Inoue, Takashi Hironaka, Toshiyuki Owaki, Duncan M. Geddes, Mamoru Hasegawa, Eric W. F. W. Alton

843
The Tupaia Paramyxovirus Envelope as Vector Delivery Module: Novel Fusion Protein Cleavage-Activation Sequence and Attachment Protein Retargeting
Christoph Springfeld, Véronika von Messling, Christian A. Tidona, Gholamreza Darai, Roberto Cattaneo

844
Determinants of Viral Tropism: A Morbillivirus V Protein Exerts Host Defense Evasion Function in T Lymphocytes
Véronika von Messling, Roberto Cattaneo

845
Properties of Sindbis Virus Vectors Produced with a Chimeric Split Helper System
Anna Ketola, Sondra Schlesinger, Jarmo Wahlfors

846
In Vivo and In Vitro Cell Targeting with Library-Selected Protease-Activatable Replication-Competent Retroviruses
Klaus Cichutek, Richard M. Schneider, Astrid Schwanitz, Yiansheng Sun, Christian J. Buchholz, Irene Hartl, Stephen J. Russell

AAV Vectors: Vector Development

847
Adeno-Associated Virus Capsids from Divergent AAV Clades and Subgroup C Adenovirus Capsids Share a Common Microtubule-Binding Mechanism Relevant to Virus Trafficking
Samir A. Kelkar, Bishnu P. De, Guangping Gao, James M. Wilson, Ronald G. Crystal, Philip L. Leopold

848
Development of AAV Vectors Targeted to Vascular Endothelium
Matthew Stackler, Jennette Crumine, Jeffrey Bartlett

849
Transduction of Mouse Dendritic Cells Is Enhanced by Repetitive Infection with Self-Complementary Adeno-Associated Virus 6 Combined with Immunostimulatory Ligands
Wayne A. Aldrich, Changchun Ren, April E. White, Shang-Zhen Zhou, Sanjay Kumar, Connie B. Jenkins, Denise R. Shaw, Theresa V. Strong, Pierre L. Triozzi, Selvarangan Ponnazhagan

850
Efficiency of Six AAV Serotypes in Transducing Rat Myocardium In Vivo
Julieta Palomeque, Jennifer A. Wellman, Shangzhen Zhou, Peter Colosi, Manuel Ramos-Kuri, Federica del Monte, Roger J. Hajjar

851
Development of Organ Homing AAV-2 Vectors
Lorraine M. Work, Stuart A. Nicklin, Nicola Britton, Michael Hallek, Hildegard Buning, Andrew H. Baker

852
Human ANP32B Protein Binds AAV-2 Rep and Increases Viral Replication and rAAV Production
Gianluca Pogoraro, Alessandro Marcello, Michael P. Myers, Mauro Giacca

853
Persistent Expression of Single Chain Antibodies Mediated by AAV5 and AAVrh.10 Vectors
Julie L. Boyer, Jeffrey Howard, Neil R. Hackett, Robert G. Peragolzi, James M. Wilson, Ronald G. Crystal

854
Optimization of AAV Serotype and Promoter for Increased Distribution of Expression in Both Somatic and Central Nervous Systems of Mice
Haiyan Fu, Joseph Muenzer, Lu Kang, Jerilynn S. Jennings, Richard J. Samuelski, Douglas M. McCarty
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855
In Vivo Spinal Cord Uptake and Neuronal Binding Properties of Tet1: A Potential Means for Enhanced Spinal Cord AAV Delivery
Thais Federici, James K. Liu, Qinghan Teng, Mary Garrity-Moses, Jun Yang, Nicholas M. Boulis

856
Transduction Patterns, Enzyme Distribution, and Vector and Enzyme Transport Resulting from Unilateral Injections of AAV 7, 8, 9, or Rh10 Expressing β-Glucuronidase into Select Brain Regions of the C3H Mouse
Cassia N. Cearley, John H. Wolfe

857
A Versatile and Efficient Viral Vector Platform for Stable In Vivo Expression of Short Hairpin RNA
Dirk Grimm, Kasum Pandey, Konrad L. Streetz, Mark A. Kay

858
Adeno-Associated Virus Vector Serotype 8 (AAV8) Achieves a Higher Level of Gene Transfer to the Central Nervous System (CNS) upon Intraventricular Injection in Neonatal Mice Compared to AAV1 and AAV2
Marike Broekman, Fabrício Costa, Comer Laryssa, LeRoy Stan, Sena Esteves Miguel

859
Enhancing Neurotropism with Multiple Mutations in the AAV-2 Capsid
Jianfeng Xu, Caroline Bass, Chunyan Ma, Ernest F. Terwilliger

860
Tissue Distribution of Expression Using AAV8-Based Vectors after Intramuscular Injection and Other Routes of Delivery
Tsuyoshi Ogura, Hiroaki Mizukami, YuanYuan Zhang, Takashi Okada, Akihiro Kume, Seiji Madoiwa, Jun Mimuro, Yoichi Sakata, Keiya Ozawa

861
AAV Gene Delivery for the Cornea
Jia Liu, Sonal S. Tuli, David C. Bloom, Gregory S. Schultz, William W. Hauswirth, Alfred S. Lewin

862
Adipose Tissue Transduction Using AAV8-Based Vectors: Inadvertent Gene Transfer into Liver
YuanYuan Zhang, Tsuyoshi Ogura, Jun Mimuro, Takashi Okada, Akihiro Kume, Yoichi Sakata, Keiya Ozawa, Hiroaki Mizukami

863
Directed Evolution of Adeno-Associated Virus (AAV) Vectors for Human Gene Therapy
Luca Perabo, Jan Endell, Susan King, Daniela Goldnau, Kerstin Lux, Michael Hallek, Hildegard Buening

864
Adsorption of Adeno-Associated Virus to Commonly Used Catheter Materials
Daisy P. Cross, Erica TenBroek, Cygini (Howie) Chan, Laura Christopherson, Orhan Soykan, Daniel C. Sigg

865
Focus on Specificity: Creating Efficient CML-Specific Recombinant Adeno-Associated Viral (AAV) Vectors Using a Random Peptide Library
Marius Stiefelhagen, Marlon R. Veldwijk, Stephanie Lauß, Jürgen A. Kleinschmidt, Frederik Wenz, W. Jens Zeller, Stefan Friehaup

866
Recombinant AAV 1, 2, 5, 8 and Chimeric 1/2-1/8 Serotypes Display Differential Transduction Efficiency after Delivery to Mouse Brain
Fabrício F. Costa, Marike Broekman, Stanley G. LeRoy, Laryssa A. Comer, Miguel Sena-Esteves

867
Use of AAV Display Libraries to Generate Myotrophic Vectors
Yu-Hung Kuo, Justin Fraser, Michael G. Kaplitt

868
Effective Purification of Adeno-Associated Virus Vectors by Disposable Ion Exchange Membranes
Takashi Okada, Mutsuko Nonaka-Sarokawa, Takayuki Ito, Takashi Matsushita, Hiroaki Mizukami, Akihiro Kume, Keiya Ozawa

869
Infectious Titer Assay for Recombinant Adeno-Associated Virus Vectors Using Direct Cell Lysis and Endpoint Taqman PCR
Simon M. Grainger, Richard Peluso, Christopher J. Gerard

870
Transfection of Mammalian Cells Using Linear Polyethylenimine Is a Simple and Effective Means of Producing Recombinant AAV
Sharon E. Reed, Elizabeth M. Staley, David J. Pintel, Gregory E. Tullis

871
rAAV2-Mediated Gene Expression in Primary Cortical Neural Cells Following Inhibition of DNA Synthesis and/or EGFR Tyrosine Kinase
Young Ran Nam, Sung Jin Kim, Chul-Hyen Joo, Yoo Kyum Kim, Heuiran Lee

Adenovirus Vectors: Vector Technologies

872
Chimpanzee Derived Adenoviral Vectors Transduce and Activate Human Dendritic Cells
Sury Somannathan, Soumitra Roy, Guangping Gao, Arbans Sandhu, James M. Wilson
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873 Adenovirus Polypeptide IIIa as a Novel Locale for Incorporation of Heterologous Peptides
Joel N. Glasgow, Elena Kashentseva, Igor P. Dmitriev, David T. Curiel

874 Long-Term Transgene Expression In Vivo from a Helper Dependent Adenovirus—Epstein-Barr Virus Hybrid Vector
Sean D. Gallaher, Jose S. Gil, Oliver Dorigo, Arnold J. Berk

875 Adenoviral Vector Targeting – New Targets for Old Capsids
Douglas E. Brough, Hinrich Staecker, Masaki Akiyama, Chi Hsu, Selva R. Murugesan, David A. Einfeld, Jason G. Gall, Lisa L. Wei, Melissa Hamilton, Duncan McVey C. Richter King

876 Development of a Novel Adenovirus-Alphavirus Hybrid Vector Providing High Efficacy and Safety in the Treatment of Liver Cancer
Min Guan, Cristian Smerdou, Juan R. Rodriguez-Madoz, M. Gabriela Kramer, Stefan Kochanek, Jesus Prieto, Cheng Qian

877 Long-Term Transgene Expression with a Second Generation Hybrid AdenoRetroviral Vector
Changyu Zheng, Weitan Zhang, Joseph M. Vitolo, Bruce J. Baum

878 Toward ‘Pseudotyping’ Adenovirus Vectors: Efficient Generation of pIX-Expressing Helper Cell Lines
Rob C. Hoeben, Taco G. Uil, Jeroen De Vrij, Leif Lindholm, Jort Vellinga

879 Transduction Enhancement by the Adenovirus/Polymer Complexes Derivatized with Protein Transduction Domain Peptides
Ilia Fishbein, Ivan S. Alferiev, Richard Gaster, Michael Chorny,Origene Nyanguile, Robert J. Levy

880 AAV-Rep 78 Mediated Rescue of a 27kb Transgene Cassette from a Helper-Dependent Ad Vector Genome
Hongjie Wang, Dmitry Shayakhmetov, Andre Lieber

881 Development of Novel E1-Complementary Cells for Adenoviral Production Free of Replication Competent Adenovirus
Deborah Farson, Lucy Tao, Derek Ko, Thomas Harding, Dominic Brignetti, De-Chao Yu, Yuanhao Li

882 Development of a Transposon-Based Approach to Identification of Novel Transgene Insertion Sites within Replicating Viral Genomes
Peter Kretschner, Fang Jin, Cecile Chartier, Paul Harden, Terry Hermiston

883 Gold Nanoparticles as an Amplifying Payload Strategy for Adenoviral Cancer Gene Therapy
Maaike Everts, Jennifer L. Leddon, Robbert J. Kok, Meredith A. Preuss, C. Leigh Millican, David E. Nikles, Duane T. Johnson, David T. Curiel

884 Integration of a Helper-Dependent Adenovirus Mediated by the Phage F6C31 Integrase
Rénald Gilbert, Laurice Desfossé, Émilie Mairey, Benoît Raymond, Michele P. Calos, Jacques P. Tremblay, Bernard Massie

885 Calcium Phosphate Coprecipitation Enhances Adenoviral Vector-Based Gene Transfer to Murine Bone Marrow Derived Dendritic Cells
Michael P. Seiler, Vincenzo Cerullo, Philip Ng, Donna Palmer, Brendan Lee

886 An Adenovirus Vector with a Chimeric Fiber Derived from Ovine Adenovirus for Novel Tropism Discovery
Masaharu Nakayama, Joel N. Glasgow, Gerald W. Both, Massimo Palmarini, Yoko Tsuruta, Kenzaburo Tani, Joanne T. Douglas, David T. Curiel

887 Reducing the Incidence of Replication Competent Adenovector by Engineering the Vector Genome
G. Campbell Kaynor, Jennifer L. Brown, Rhonda Brown, Xinzhong Wang

888 Optimization of Adenovirus Serotype 35 Vectors for Efficient Transduction in Human Hematopoietic Progenitors; Comparison of Promoter Activities
Fuminori Sakurai, Hiroki Mizuguchi, Kenji Kawabata, Tetsuhide Yamaguchi, Takao Hayakawa

889 Lanthanum Enhances Adenovirus-Mediated Gene Transfer
Oliver B. Betz, Glyn D. Palmer, Volker M. Betz, Keith Crawford, Christopher Niyibizi, Mark S. Vrahos, Christopher H. Evans
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890 Development of a Single Vector System for Gene Delivery Via Hybrid Helper Dependent Adenovirus - Epstein-Barr Virus Vector for Long Term Persistence
Jose S. Gil, Sean D. Gallaher, Oliver Dorigo, Arnold J. Berk

891 A Flexible System for Ad35-Based Vector Generation
Wentao Gao, Angela Montecalvo, Huijie Sun, Paul D. Robbins, Andrea Gambotto

892 GeneJammer® Enhances Cellular Transduction with Recombinant Adenovirus
Christine M. Dilling, Pablo Bosh, Alan R. Davis, Jessica A. Shafer, Steven Stice, Malavosklish Bikram, Jennifer L. West, Elizabeth A. Omlsted-Davis

893 Using QPCR and Automation to Assign Infectious Potencies to Adenovirus Based Vaccines and Vectors for Gene Therapy
Alan C. Puddy, Jenny Xu, Amy M. Bowman, Yahua Zhang, Charles Y. Tan, Timothy L. Schofield, Peter A. DePhillips, John A. Lewis, Janyathi J. Wolf

Inborn Errors of Metabolism: Liver and Pancreatic Diseases

894 A Novel Mechanism between Type II Diabetes Mellitus and Procalcitonin Gene Expression
Mehment Ali Soylemez, Oktay Seyment, Gunnur Yigit

895 Ex Vivo Gene Therapy of Metabolic Liver Disorders Using Uncultured and Lentivirally Transduced Hepatocytes
Tuan Huy Nguyen, Jacques Birraux, Barbara Wildhaber, Catherine Fux, Francois Trivin, Anne Myara, Claude Lecoultre, Didier Trono

896 Complete and Permanent Correction of Hyperbilirubinemia in Newborn Gunn Rats by Lentiviral-Mediated Gene Transfer
Tuan Huy Nguyen, Marta Bellodi-Privato, Dominique Aubert, Anne Myara, Didier Trono, Nicolas Ferry

897 Recombinant AAV2/8 Vector-Mediated Gene Therapy Corrects Hyperphenylalaninemia in Murine PKU
Cary O. Harding, Melanie B. Gillingham, Kelly Hamman, Andrew Bird, Dwight Koeler

Liver-Directed, AAV- and Lentivirus-Mediated Gene Therapy in the Phenylketonuria Mouse Model Pah-enn2
Zhaobing Ding, Panco Georgiev, Beat Thony

Glucose-Stimulated Insulin Secretion from an Engineered Human Liver Cell Line Is Regulated by ATP-Sensitive Potassium Channels and the L-Type Calcium Channel
Ann M. Simpson, Edwin Ch'ng, Guo J. Liu, Chang Tao, Donald K. Martin, Mark Lutherborrow, Bernard E. Tuch

Overcoming Gender-Specific Barrier of AAV-Mediated Gene Therapy by Neonatal Intervention
Akihiro Kume, Tsuyoshi Ogura, Hiroaki Mizukami, Shigeo Kure, Takashi Okada, Masashi Urabe, Takashi Matsushita, Keiya Ozawa

Recovery from Type 1 Diabetes by Insulin and Glucokase Expression in Skeletal Muscle
Joel Montane, Alex Mas, Xavier M. Anguela, Anne M. Douar, Pedro J. Otaegui, Fatima Bosch

In Toto Gene Correction by Meganuclease-Induced Homologous Recombination in Mouse Liver
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1022 Single-Dose Intravenous Toxicity Study with the Oncolytic Adenovirus Vector VRX-007, Adenovirus Type 5, and a Replication-Defective Adenovirus in Syrian Hamsters Karoly Toth, Jacqueline F. Spencer, Drew L. Lichtenstein, Ann E. Tollefson, Jennifer M. Meyer, Maria A. Thomas, Mohan Kuppuswamy, Baoling Ying, Louis A. Zumstein, William S. M. Wold


1024 An Oncolytic Conditionally Replicating Adenovirus for Hormone-Dependent and Hormone-Independent Prostate Cancer Wing-Shing Cheng, Helena Dzojic, Berith Nilsson, Thomas H. Totterman, Magnus Essand

1025 A PPT Promoter-Based Adenovirus with Two-Step Transcriptional Amplification (TSTA) for Experimental Prostate Cancer Helena Dzojic, Wing-Shing Cheng, Magnus Essand

1026 In Vivo Imaging for Evaluation of Adenoviral Capsid Modifications for Systemic Delivery in an Orthotopic Model of Locally Advanced Lung Cancer Merja Sarkioja, Anna Kanerva, Tanja Hakkarainen, Jarmo Salo, Lotta Kangasniemi, Mari Raki, Tiitu Ranki, Hongju Wu, David T. Curiel, Akseli Hemminki


1028 Reduction of Replication of Oncolytic Adenoviruses with Pharmacological Intervention: Treatment of Replication Associated Toxicity Anna Kanerva, Mari Raki, Lotta Kangasniemi, Renee A. Desmond, Helena Isoniemi, Krister Hockerstedt, Akseli Hemminki

1029 Integrin and Serotype 3 Receptor-Targeted Virotherapy Using a Telomerase-Selective Oncolytic Adenovirus Yosuke Kauwakami, Angel A. Rivera, Minghui Wang, Dirk M. Nettelbeck, Mariam A. Stoff-Khalili, Mack N. Barnes, Gene P. Siegal, Ronald D. Alvarez, David T. Curiel

(Note: Abstract 328 has been moved from Poster Session I to Poster Session III and will follow Abstract 1029. See page 65 for abstract title and authors.)

Hematologic - New Advances

1030 A Rhesus Macaque Transplant Model for Drug Selection of Hematopoietic Stem Cells Transduced with MGMT Vectors Yan Shou, John Gray, Brian Agricola, Zhijun Ma, Derek A. Persons, Brian P. Sorrentino


1032 Hepatocyte Growth Factor Maintains Hematopoietic Progenitors during Retroviral Transduction Todd E. Meyerrose, Phillip E. Herrbrich, Michael H. Creer, Ian A. Nolta
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1033
Enhanced Engraftment of Retrovirally Transduced Hematopoietic Stem Cells Following Treatment with Diprotin-A
Chaurui Tian, Jessamyn Bagley, Daron Forman, John Iacomini

1034
Automated Retroviral Insertion Site Sequence Analysis and Mapping Tool followed by Database Analysis
Stephanie Laufs, Frank A. Giordano, Agnes Hotz-Wagenblatt, Uwe Appelt, Daniel Lauterborn, Kurt Fellenberg, W. Jens Zeller, Stefan Fruehlauf

1035
Use of Multi Color p27kip1 Based Flow Cytometric Assay To Evaluate and Characterize G0 to G1 Cell Cycle Related Changes in Human Hematopoietic Cells after Subjection to a Clinically Applicable Ex Vivo Transduction Protocol
Jesper Bonde, Ryan Lahey, Phillip Herrbrich, Jan A. Nolta

1036
Engraftment of side population Stem Cells in Canine Recipients
Andrea K. Vaags, David Caton, Krista Halling, Cathy Gartley, Suzana Rosic-Kablar, Stephen A. Kruth, Margaret R. Hough

1037
Adherent Human Umbilical Cord Blood Derived Mesenchymal Stem Cells Do Not Augment Viral Gene Transfer into Hematopoietic Stem/Progenitor Cells
Todd E. Meyerson, Phillip E. Herrbrich, Michael H. Creer, Jan A. Nolta

1038
Lentiviral Gene Transfer into HSC Is Enhanced by Early-Acting Cytokines without Impairing Stem Cell Properties and Involves Cellular Responses Distinct from Cell Cycle Control
Francesca Santoni de Sio, Luigi Naldini

1039
Ex Vivo Pre-Selection Increases Marking Following Transplant of Mixtures of Bone Marrow from Wild Type and Green Fluorescent Protein-Methylguanine Methyltransferase P144K Fusion Protein Transgenic Mice
Uimook Choi, Lanise Cardwell, Natalia Loktionova, Harry L. Malech

1040
Donor Origin of Multipotent Adult Progenitor Cells in Radiation Chimeras
Morayma Reyes, Sheng Li, En Kimura, Jeffrey S. Chamberlain

1041
Delayed Infusion of Hematopoietic Stem Cells Improves Engraftment after Low-Dose Parenterally Administered Busulfan in a Congenic Murine Transplant Model
Matthew M. Hsieh, Saskia Langemeijer, Bob Wesley, Elizabeth M. Kang, John F. Tisdale

1042
Creation of Tolerogenic Dendritic Cells by Non Viral Gene Transfer with Immunoliposomes
Peng H. Tan, Sven C. Beutelspacher, Giovanna Lombardi, Andrew J. T. George

1043
In Vivo Gene Transfer into Rat Hematopoietic Stem Cells Using rSV40 Viral Vectors
Bianling Liu, Jean-Pierre Louboutin, Judy Daviau, David S. Strayer

1044
Radioprotective Gene Therapy through Retroviral Expression of Manganese Superoxide Dismutase
Thomas D. Southgate, Victoria Sheard, Michael D. Milson, Rob J. Mairs, Marie Boyd, Leslie J. Fairbairn

1045
Overexpression of an ABCG2 Multidrug Transporter Variant Does Not Effect Proliferation or Differentiation of Hematopoietic Stem Cells
Olga Ujhelly, Nora Kucsmá, Judith Cervenak, Linping Chen, Manuel Grez, Balazs Sarkadi, Katalin Nemet

1046
Stable Correction of Hematopoietic Stem Cells with Non-Viral Gene Transfer
Meenakshi Noll, Ryan Bennett, Stephen Yant, Mark A. Kay, Markus Groppme

1047
Chemoprotective Gene Therapy through Retroviral Expression of the X-Ray Cross Complementing Protein (XRCC1)
Thomas D. Southgate, Katherine Clarke, Leslie J. Fairbairn

1048
Engineering and Development of I-Anil Homing Endonucleases for Gene Correction Applications
Andrew M. Scharenberg, David J. Rawlings, Raymond J. Monnat, Barry L. Stoddard

1049
Serum Free Fedia Is the Best for Cord Blood Hematopoietic Cells Expansion
Kamran Alimoghaddam, Mitra Khalili, Masoud Soleimani, Lili Moezi, Ardisbir Ghavamzadeh
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Gene Regulation: Regulated Systems and Tissue Specific Expression
1050
Adenoviral Clostridial Light Chain Expression in the Medial Forebrain Bundle: A Reversible Model of Dopamine Depletion
Mary E. Garrity-Moses, Qingshan Teng, Jun Yang, Thais Federici, Erin Gilbert, Thiyagarajan Subramanian, Nicholas M. Boulis

1051
Prostate Cancer-Targeted Suicide Gene Therapy Achieved Effective Tumor Destruction While Safeguarding Against Systemic Toxicity
Mai Johnson, Makoto Sato, Burton Jeremy, Sanjiv S. Gambhir, Michael Carey, Lily Wu

1052
A Versatile Chimeric Enhancer Vector That Is Highly Inducible by Hypoxia and Metals

1053
Novel Exogenously Regulated Stem Cell–Mediated Gene Therapy Platform for Spinal Fusion
Gadi Pelled, Amir Hasharoni, Yoram Zilberman, Gadi Turgeman, Gregory A. Helm, Meir Libergall, Dan Gazit

1054
Bio-Distribution Studies of Lentiviral Vectors Injected Intravenously by Dynamic Bioluminescence Imaging Analyses: Development of APC-Transcriptionally Targeted Vectors for Genetic Vaccination Purposes
Takahiro Kimura, Richard C. Koya, Nori Kasahara, Renata Stripecke

1055
Reduced Toxicity Selection of Modified Murine Repopulating Stem Cells Results in an Unchanged Differentiation Potential of Oligoclonal MGMT-P140K Overexpressing Murine Hematopoiesis
C. Ball, M. Schmidt, C. von Kalle, H. Glimm

1056
Suppression and Enhancement of Gene Repair by the Modulation of ATM Function in Mammalian Cells
Julia Engstrom, Luciana Ferrara, Hetal Parekh-Olmedo, Eric B. Knice

1057
Generating Efficient Promoters for Cardiovascular Gene Therapy Using Synthetic Promoter Libraries
Stuart A. Nicklin, Anna F. Dominiczak, Andrew H. Baker
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SATURDAY, JUNE 4

1067
Regulated Expression of Biotherapeutic Proteins and shRNA Using an Ecdysone Receptor-Based Gene Switch
Prasanna Kumar, Anand K. Katakam, Chris L. Herold

1068
Luciferase as an In Vivo Reporter for Selective Liver Repopulation in Adult FAH-/- Mice
Kirk J. Wangensteen, Andy Wilber, Guisheng Zeng, Joel L. Frandsen, R. Scott McIvor, Stephen C. Ekker, Xin Wang

1069
Increased Antioxidant Levels Following MnSOD-PL Transfection Results in Increased Radioprotection of Normal Tissue but Not Tumor Cells
Michael W. Epperly, Suhua Nie, Xichen Zhang, Joel S. Greenberger

1070
Easy Search on Clinical Gene Transfer: The Euregenethy (EC-DG Research FP5-TN) Data Base on Pre-Clinical & Clinical Gene Transfer & Therapy
Odile Y. D. Cohen-Haguenauer, Fatima Bosch, Manuel Carrondo, Klaus Cichutek, Nicole Deglon, Barbara Demeneix, George Dickson, Gosta Garthon, Bernd Gansbacher, Cecilia Melani Luigi Naldini, Felicia Rosenthal, Sandro Russoni, Seppo Yla-Herttuala, Heinz Zwierzina

1071
Potential Utility of Adipose-Derived Stromal Cells (ASCs) as a Carrier for Ex Vivo Gene Therapy – Gene Transfer Efficiency of ASCs
Rei Ogawa, Hideki Hanawa, Yukihiko Hirai, Toshiyuki Kurai, Hiroshi Mizuno, Hiko Hyakusoku, Takashi Shimada

1072
Superactivation of the SRαΔ and CMV Promoters Using Designed Zinc Finger Proteins
Victor V. Bartsevich, Andreas Reik, Yuanqin Zhou, Michelle Kong, Xiao-Yong Li, Lyndon Warfe, Lei Zhang, Casey C. Case, Trevor Collingwood, Philip D. Gregory Edward J. Rebar

1073
Down-Regulation of Epithelial Glycoprotein-2 and VEGF-D by Engineered Zinc Finger Protein Transcription Factors

SUNDAY, JUNE 5

Oral Abstract Session 510
Clinical Gene Therapy
Room: 240 Complex

1074 10:15 AM
The Use of Genetically Modified Antigen Presenting Cells to Generate Donor-Derived Virus-Specific Cytotoxic T Lymphocytes Reactive Against CMV and Adenoviral Antigens for Clinical Use
Gary D. Myers, Ann Leen, Helen Huls, Elizabeth Buza, Jeff Chang, Robert A. Krance, George Carrum, Malcolm K. Brenney, Cliona M. Rooney, Helen E. Heslop Catherine M. Bollard

1075 10:30 AM
Phase I Clinical Trial Demonstrates Safety and Feasibility of Autologous Cellular Therapy with Lentiviral Modified CD4 T Cells Expressing Anti-HIV Antisense in Patients with HAART Resistant HIV-1 Infection
Boro Dropulic, Rob-Roy MacGregor, Tessio Rebello, Peter Manilla, Laurent Humeau, Xiaolin Lu, Vladimir Slepukhin, Gwendolyn K. Binder, Cathy Ybarra, Bruce L. Levine Frederic Bushman, Carl H. June

1076 10:45 AM
Stable Polyclonal Hematopoietic Repopulation after Successful Clinical Gene Therapy of Chronic Granulomatous Disease (CGD)
Manfred Schmidt, Kerstin Schwarzwelder, Marion G. Ott, Stefan Stein, Hanno Glimm, Annette Deichmann, Ulrich Sieler, Dieter Hoelzer, Reinhard Seger, Manuel Grez Christof von Kalle

1077 11:00 AM
Phase I Clinical Trial of Recombinant Adeno-Associated Virus (rAAV)-Alpha-1 Antitrypsin Vectors
Mark L. Brandly, Margaret Humphries, Sihong Song, Thomas Conlon, Amy Poirier, Barry J. Byrne, Richard Snyder, Terence R. Flotte

1078 11:15 AM
Therapeutic Misconception in Early Phase Gene Transfer Trials

1079 11:30 AM
Successful Myoblast Transplantation to Duchenne Muscular Dystrophy Patients
Jacques P. Tremblay, Daniel Skuk, Sylvain Michel, Raynald Roy, Jean-Guy Lachance, Hélène Senay, Deshènes Louise, Goulet Marlyne, Brigitte Roy, Jean-Pierre Bouchard
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SUNDAY, JUNE 5

Oral Abstract Session 511
AAV Vectors: Disease Applications
Room: 260/264

1080 10:15 AM
rAAV Delivered Ribozyme Targeting Mouse Opsin to Rescue Vision in P23H Rats
Marina S. Gorbatyuk, Adrian M. Timmers, Pang Jijing, William W. Hauswirth, Alfred S. Lewin

1081 10:30 AM
Sustained Correction of Disease in Naïve and AAV2-Pretreated Hemophilia B Dogs Using Pseudotyped AAV Vectors
Lili Wang, Roberto Calcedo, Timothy C. Nichols, Inder M. Verma, James M. Wilson

1082 10:45 AM
Transduction Comparison of AAV Serotype Vectors in the Central Nervous System
Sharon K. Reimsnider, Fredric Manfredsson, Nick Muzyczka, Ronald J. Mandel

1083 11:00 AM
Systemic rAAV-CB-IL-10 Expression and Its Long Term Effects on CD4+CD25+ T Cell Populations
Christian Muller, Sofia A. Braag, Ashley Martino, Mark A. Atkinson, Terence R. Flotte

1084 11:15 AM
A Screen for Host Cellular Proteins That Interact with Adeno-Associated Virus Capsid Proteins
Bassel Akache, Daria Glazer, Julie Park, Mark A. Kay

1085 11:30 AM
Directed Evolution of AAV2 Leads to Improved Viral Function
James T. Koerber, Narendra Maheshri, Brian Kaspar, David V. Schaffer

1086 11:45 AM
Patterns of Gene Expression from In Utero Delivery of AAV Serotype 1
Roberto Bilbao, Daniel P. Reay, Juan Li, Xiao Xiao, Paula R. Clemens

1087 12:00 PM
Adeno-Associated Virus-Mediated Transduction of VEGF165 Markedly Improves Functional Recovery of Infarcted Myocardium in Chronically Instrumented Dogs
Matteo Ferrarini, Nikola Arsic, Serena Zacchigna, Fabio Recchia, Lorena Zentilin, Mauro Giacca, Thomas Hintze

Oral Abstract Session 512
Adenovirus Vectors: Adaptive Immunity and Vaccines
Room: 120/124

1088 10:15 AM
Antigen Epitopes Expressed in Different Adenovirus Capsid Proteins Induce Different Levels of Epitope-Specific Immunity
Anja Krause, Stefan Worgall, Ju H. Joh, Neil R. Hackett, Peter W. Roelvink, Joseph T. Bruder, Thomas J. Wickham, Imre Kovacs, Ronald G. Crystal

1089 10:30 AM
Examine the Cross-Reactivity of Vector-Specific T Cells between Human Adenoviral H5 and Simian Adenoviral C7 Vector in Mice
Yan Zhi, Di Wu, Heather Jordan, Guangping Gao, James M. Wilson

1090 10:45 AM
Adenovirus Fiber Incorporates an Immunodominant MHC Class I Restricted Epitope Peptide
Jian Chen, Allan J. Zajac, Xin Xu, Ping Ar Yang, Qi Wu, Kohtaro Fujishashi, Hui-Chen Hsu, John D. Mountz

1091 11:00 AM
Analysis of T-Cell Responses to Helper-Dependent Serotype 5-Based Capsid-Modified Adenovirus (HD.5/35) or First-Generation Serotype 11-Based (Ad11) Vectors
Nelson C. Di Paolo, Daniel Stone, Andre Lieber

1092 11:15 AM
Adenovirus-Based Vaccine Prevents Pneumonia in Ferrets Challenged with the SARS Coronavirus
Gary P. Kobinger, Thomas Rowe, Guangping Gao, Joanita M. Figueredo, Julio Samniguel, Peter Bell, Robert J. Hogan, James M. Wilson

1093 11:30 AM
An Adenovirus-Retrotransposon Hybrid Vector Achieves Stable Transformation of Quiescent and Primary Human Somatic Cells
Shuji Kubo, Harris S. Soifer, Marie del Carmen Selene, John V. Moran, Haig H. Kazazian Jr., Noriyuki Kasahara

1094 11:45 AM
A Novel Replication Competent, but Packaging Deficient Adenoviral(Ad) Vector [E1+, 100K-]hGAA for High Level hGAA Expression and Reduced Toxicity in Gene Therapy of Glycogen Storage Disease II (GSD-II)
Fang Xu, Zachary Hartman, Delila Serra, Andrea Amalfitano

1095 12:00 PM
Silica Gel in Targeted and Controlled Viral Gene Therapy
Mika K. Koskinen, Mervi Toriseva, Mika J. Jokinen, Harry Jalonen, Jukka I. Salonen, Veli-Matti Kälhäri
ABSTRACTS

SUNDAY, JUNE 5

Oral Abstract Session 513
Naked DNA Gene Transfer: Chromosomal Integration
Room: 263/267

1096 10:15 AM
Physical Determinants for Efficient Hydrodynamic Gene Delivery to Liver
Takeshi Suda, Regis Vollmer, Dexi Liu

1097 10:30 AM
Complete and Persistent Phenotypic Correction of Phenylketonuria in Mice by Site-Specific Genome Integration of Phenylalanine Hydorxylase cDNA
Li Chen, Savio L. C. Woo

1098 10:45 AM
Duration of Expression of Sleeping Beauty Transposase by Hydrodynamic Injection of C57/BL6 Mice
Jason B. Bell, Elena L. Aronovich, Beth E. Larson-DeBruzzi, Brenda L. Koniar, Roland Gunther, Chester B. Whitley, R. Scott McIvor, Perry B. Hackett

1099 11:00 AM
Beyond LoxP: Defining Functional Lox-Like Sequences for the Cre Recombinase
Jamie E. Sheren, Elizabeth Ketner, Jarred Keith, Stephen J. Langer, Leslie A. Leinwand

1100 11:15 AM
Site-Specific Transposon Integration
Joseph M. Kaminski, Maragatha Vally, Todd Tenenholz, James B. Summers, Craig J. Coates

1101 11:30 AM
Fusion Proteins Consisting of the Sleeping Beauty Transposase and the Polydactyl Zinc Finger Protein E2C Direct Transposon Integration into a Unique Human Chromosomal Sequence
Stephen R. Yant, Yong Huang, Mark A. Kay

1102 11:45 AM
Stable, Non-Viral Gene Transfer to Human CD34+ Hematopoietic Progenitor Cells Using the Sleeping Beauty Transposon
Sarah Nightingdale, Roger P. Hollis, Karen Pepper, Donald B. Kohn

1103 12:00 PM
Site-Specific Chromosomal Integration in Human Mesenchymal Stem Cells Mediated by PhiC31 Integrase
Annahita Keravala, Juergen Hoelters, Sohail Jarrahian, Thomas W. Chalberg, Marisa Ciccarella, Peter Neth, Michele P. Calos

Oral Abstract Session 514
Molecular Conjugates: Vector Engineering
Room: 265/266

1104 10:15 AM
Imaging of sec-R Directed and PEG-Stabilized Gene Transfer Nanoparticles in CF Mice
Maxwell Kotlarchyk, Zhenghong Lee, Mark Cooper, Pamela B. Davis, Assem G. Ziady

1105 10:30 AM
DQAsomes Deliver Mitochondrial Leader Peptide Conjugates of Plasmid and Linear Double-Stranded DNA into Mitochondria within Living Mammalian Cells
Gerard G. M. D’Souza, Sarathi V. Bodlapati, Robert N. Lighthourlers, Volkermar Weissig

1106 10:45 AM
Development and Testing of Ternary Peptide Copolymerized Gene Delivery Systems
Changpo Chen, Ji-seon Kim, Dijie Liu, Marie McMuff, Kevin G. Rice

1107 11:00 AM
Cellular Internalization and Distribution of DNA Complexes from Cell-Adhesive Substrates
Zain Bengali, Lonnie D. Shea

1108 11:15 AM
Ad5 Penton Base Recombinant Protein: Intracellular Trafficking & Implications for Gene Delivery
Altan Rentsendorj, Jiansong Xie, Michelle MacVeigh, Hasmik Agadjanian, Sarah F. Hamm-Alvarez, Lali K. Medina-Kauwe

1109 11:30 AM
Single-Cell Observations of Intracellular Gene Delivery Particles: pH Buffering and Trafficking
Swaroop Mishra, Rajan P. Kulkarni, Scott E. Fraser, Mark E. Davis

1110 11:45 AM
Selective Chemical Modification on Polyethylenimine and Its Effects on Transfection Efficiency and Cytotoxicity
Xiang Gao, Dexi Liu

1111 12:00 PM
TAT-Streptavidin: A Novel Drug Delivery Vector for the Intracellular Uptake of Macromolecular Cargo
Brian Albarran, Richard Tö, Patrick Stayton
ABSTRACTS

SUNDAY, JUNE 5

Oral Abstract Session 515
Cardiovascular: Angiogenesis and Vascular Gene Therapy
Room: 261/262

1112 10:15 AM
The Treatment with Intra Muscular (IM) Vascular Endothelial Growth Factor (VEGF) Gene Compared with Placebo for Patients with Diabetes Mellitus and Critical Limb Ischemia (CLI), a Double Blind Phase III Study

1113 10:30 AM
Spliceosome Mediated RNA Trans-Splicing (SMART™) Strategy To Increase the Production of Human Apolipoprotein (ApoA1)
M. Puttaraju, Jun Wang, Alan T. Remaley, Bryan H. Brewer, Jr, Gerard J. McGarrity, Mariano A. Garcia-Blanco

1114 10:45 AM
Antithrombotic Effects of Endogenous Expressed Activated Protein C (aPC) in Murine Models
Joerg Schuettrumpf, Alexander Schlachterman, Jianxiang Zou, Jianhua Liu, Christian Furlan Freguia, Valder R. Arruda

1115 11:00 AM
Apo A-I and ABCA1 Gene Transfer both Increase HDL Cholesterol but Have Opposing Effects on Progression of Early Atherosclerosis in apo E Deficient Mice
Joke Lievens, Jan Snoeys, Yingmei Feng, Desére Collen, Bart De Geest

1116 11:15 AM
Gene Transfer of SERCA2a Inhibits Smooth Muscle Proliferation and Reduce Neointima Formation after Balloon Injury in the Rat
Larissa Lipskaia, Federica Del Monte, Roger J. Hajjar, Anne-Marie Lompre

1117 11:30 AM
Gene Therapy in a Rat Model of Pulmonary Hypertension
Stacy L. Porvasnik, Tankut Onal, Yoshi Sakai, Raquel Torres, Glenn A. Walter, Barry J. Byrne, Carolyn T. Spencer

1118 11:45 AM
A Skin Gene Therapy Approach to Treat Hypertension Using Long-Term Expression and Systemic Delivery of Atrial Natriuretic Peptide (ANP) from the Skin
Jean-Philippe Therrien, Christine L. Teck, Manabu Ohyama, Jonathan C. Vogel

1119 12:00 PM
Isolation of a Novel Therapeutic Angiogenic Peptide, AG-30, by High Throughput Screening Using the HVJ-E Vector
Tomoyuki Nishikawa, Hironori Nakagami, Akito Maeda, Katsuto Tamai, Yasufumi Kaneda

Oral Abstract Session 516
Muscle and Connective Tissue: Gene Therapy for Connective Tissue
Room: 230/231

1120 10:15 AM
Use of Gene Activated Muscle Grafts for the Repair of Large Bone Defects
Volker M. Betz, Oliver B. Betz, Ara Nazarian, Carmencita G. Pilapi, Mary L. Bouxsein, Louis C. Gerstenfeld, Thomas A. Einhorn, Mark S. Vlahos, Christopher H. Evans

1121 10:30 AM
Osteogenic Growth Peptide Preferentially Enhances Trabecular Bone Mass
Todd E. Meyerrose, Elisheva Smith, Jan A. Nolta, Baruch Frenkel

1122 10:45 AM
Transgenic Vasoactive Intestinal Peptide Shows Local Disease Modifying Effects in a Murine Model of Sjögren’s Syndrome
Beatrijs M. Lodde, Fumi Mineshiba, Jianghua Wang, Ana P. Cotrim, Sandra Afione, Paul P. Tak, Bruce J. Baum

1123 11:00 AM
Adenovirus-Mediated Expression of Sonic Hedgehog Increases the Number of CD34+ Cells in the Epidermal Stem Cell Niche in Hair Follicles
Howard Lou, Andrey Punteleyev, Angela M. Christiano, Ronald G. Crystal, Philip L. Leopold

1124 11:15 AM
Correction of Glycogen Storage Disease Type II by an AAV Vector Encoding Highly-Secreted, Chimeric Acid a-glucosidase
Baodong Sun, Talmage Brown, Andrew Bird, Ayn Schneider, Sarah P. Young, Alison McVie-Wylie, Y.-T. Chen, Dwight D. Koeberl

1125 11:30 AM
AAV-Mediated Myostatin Propeptide Gene Delivery Results in Growth and Hypertrophy of Skeletal but Not Cardiac Muscles
Chumping Qiao, Jianbin Li, Tong Zhu, Chunlian Chen, Terry O’Day, Jon Watchko, Juan Li, Xiao Xiao

1126 11:45 AM
Further Characterization of Msx1 De-Differentiated Cells
Qiang Liu, Wen Liu, Yaming Wang
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1127 12:00 PM
Perivascular CD45-:Sca-1+:CD34- Cells Are Derived from Bone Marrow Cells and Participate in Dystrophic Skeletal Muscle Regeneration
Sheng Li, Morayma Reyes, En Kimura, Jessica Foraker, Miki Hagakura, Leonard Meuse, Brent Fall, Jeffrey S. Chamberlain

Oral Abstract Session 517
Cancer - Immunotherapy: Genetic Strategies to Induce and Augment Anti-Tumor Immunity
Room: 123/127

1128 10:15 AM
Ad-sig-TAA/ecdCD40L Vector Prime, TAA/CD40L Protein Boost Vaccine Breaks Tolerance for Tumor Associated Antigens (TAA) and Suppresses Growth and Metastasis of Epithelial Neoplastic Cells
Yucheng Tang, Jonathan Maynard, Xiang Ming Fang, Wei Wei Zhang, Albert Deisseroth

1129 10:30 AM
Ad.Interferon Beta Is an Effective Treatment in an Orthotopic Model of Broncho-Alveolar Non-Small Cell Lung Cancer Via NK Cell and CD 8+ T Cell Mechanisms
Michael J. Wilderman, Jin Sun, Anil Vachani, Eiji Suzuki, Dan H. Sterman, Larry R. Kaiser, Steve M. Albelda

1130 10:45 AM
A Single-Component CD40-Targeted Adenoviral Vector for Efficient Transduction and Activation of Cutaneous Dendritic Cells
Nikolay Korokhov, Sam C. Noureddini, David T. Curiel, Saskia J. A. M. Santegoets, Rik J. Scheper, Tanja D. de Gruijl

1131 11:00 AM
Generation of Whole Cell Vaccines for Acute Myeloid Leukaemia by Lentivirus Mediated IL-2/CD80 Transduction
Lucas Chan, Nicola Hardwich, Dave Darling, Joop Gaken, Joanna Galea-Lauri, Steve Devereux, Ghulam Mufti, Farzin Farzaneh

1132 11:15 AM
Role of Akt in Dendritic Cell (DC) Survival and Application of an Akt “LIFE SWITCH” in DC-Based Tumor Vaccines
Dongsu Park, Mamatha R. Seethammagari, David M. Spencer

1133 11:30 AM
A Phase I Study of Antitumor Vaccination Using Genetically Modified Tumor Cells Expressing α(1,3)Galactosyltransferase in Patients with Refractory or Recurrent Non-Small Cell Lung Cancer (NSCLC): Preliminary Results
John C. Morris, John E. Janik, Nicholas Vahanian, Leslie Moses, Diana O’Hagan, Lucinda Tennant, Stefania Pittaluga, Wendy Gao, Paul Albert, Tatiana Seregina Charles J. Link

1134 11:45 AM
Intratumoral Injection of Dendritic Cells Engineered to Secrete Interleukin-12 by Recombinant Adenovirus in Patients with Metastatic Gastrointestinal Carcinomas
Guillermo D. Mazzolini, Carlos Alfaro, Bruno Sangro, Esperanza Feijoo, Juan Ruiz, Alberto Benito, Inigo Tirapu, Ainhoa Arina, Josu Sola, Maite Herraz Felipe Lucena, Cristina Olague, Jose Subtil, Jorge Quiroga, Ignacio Herrero, Belen Sadaba, Maurizio Bendandi, Cheng Qian, Jesus Prieto, Ignacio Meler

1135 12:00 PM
Results of a Phase I/II Clinical Trial with OncoVEXTM-GM-CSF, a Second Generation Oncolytic Herpes Simplex Virus
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Fax: 508-616-0476
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www.bioprocessintl.com
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**DNAVEC Corporation**
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5-13-1 Toranomon, Minato-ku, Tokyo 105-0001
JAPAN
Phone: +81-3-5403-7061
Fax: +81-3-5403-7062
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Kemp Biotechnologies, Inc.
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Frederick, MD 21704
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E-Mail: b.miller@kempbiotech.com
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505 S. Rosa Road, Suite 104
Madison, WI 53705
Phone: 608-441-2824
Fax: 608-441-2849
E-Mail: Claire.ruzicka@mirusbio.org
www.mirusbio.com
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Phone: 760-918-0007
Fax: 760-918-0076
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www.molecularmed.com
Booth Number: 315
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EXHIBITOR DESCRIPTIONS

National Gene Vector Laboratories
650 Barnhill Drive, MS 205
Indianapolis, IN 46202-5120
Phone: 317-274-0448
Fax: 317-274-4518
E-Mail: kwicker@iupui.edu
www.ngvl.org
Booth Number: 215
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.

Nature Technology Corporation
4701 Innovation Drive
Lincoln, NE 68521
Phone: 402-472-6530
Fax: 402-472-6532
E-Mail: natx@natx.com
www.natx.com
Booth Number: 604
Nature Technology Corporation (NTC) is a full-service vector development company, specializing in the production of plasmid DNA and recombinant proteins for research and preclinical use. NTC’s pDNA fermentation process currently produces DNA at >1g/L, leading the industry in quality and economy: http://www.natx.com.

Office of Biotechnology Activities, National Institutes of Health
6705 Rockledge Drive, Suite 750
Bethesda, MD 20892
Phone: 301-496-9838
Fax: 301-496-9839
E-Mail: bm128w@nih.gov
www4.od.nih.gov/oba
Booth Number: 205-207
The NIH Office of Biotechnology Activities (OBA) will be exhibiting its 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 Genetic Modification Clinical Research Information System (GeMCRIS), as well as an extensive, Web-based resource on informed consent in gene transfer research.

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Phone: 631-844-5051
Fax: 631-844-5112
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EXHIBITOR DESCRIPTIONS

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Huntsville, AL 35806
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Fax: 256-704-4849
E-Mail: info@openbiosystems.com
www.openbiosystems.com
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Booth Number: 308
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SeqWright, Inc.
2575 W. BellFort, S-2001
Houston, TX 77054
Phone: 713-528-4363
Fax: 713-528-6232
E-Mail: erhodes@sangamo.com
www.seqwright.com
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Memphis, TN 38105
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Fax: 631-253-5445
www.vivascience.com
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May 31 - June 4, 2006