In Vivo Barriers to Gene Delivery [2007]

Session 1 MONITORING GENE DELIVERY AND EXPRESSION

MONDAY 11/26/2007, 7:30 PM

M. Barry / P. Leopold

#	lname	Title	Talk Length
1	Barry	Tracking cell targeting ligands and vectors by optical imaging	15
2	Leopold	Overcoming barriers to successful *CFTR* gene delivery and expression for cystic fibrosis	15
3	Buyens	Monitoring complex integrity of siRNA containing nanoparticles in serum using fluorescence fluctuation spectroscopy	10
4	Loo	Tracking the fate of chitosan-DNA nanoparticles in a rodent closed intestinal loop model	10
5	Deol	Levels of utrophin determine extent of correction of the dystrophic phenotype following helper-dependent adenovirus delivery	10
6	Mueller	Direct comparative analysis of rAAV1 and rAAV5 pseudotyped vectors using aerosol delivery of firefly and renilla luciferase reporters co-delivered to the lungs of chimpanzees	10

Session 2 MISLOCALIZATION, SEQUESTRATION AND TARGETING

TUESDAY 11/27/2007, 9:00 AM

L. Seymour / S. Russell

#	lname	Title	Talk Length
7	Seymour	Polymer cloaking of adenovirus particles for improved delivery	15
8	Russell	Targeting CD46—How can a ubiquitous receptor be a good target for cancer therapy?	15
9	Duerner	In vivo selection of retroviral antibody display libraries for tumor homing	10
10	Funke	Cell entry targeting of lentiviral vectors through pseudotyping with the measles virus H and F proteins	10
11	Kelly	MicroRNA-mediated targeting of an oncolytic picornavirus	10
12	Peng	Tumor neovessel targeted oncolytic MVs	10
13	Thacker	In vivo adenovirus-mediated dendritic cell transduction for melanoma therapy	10
14	Asokan	Rational engineering of chimeric AAV vectors with altered receptor usage and tissue tropisms in vivo	10
15	Barry	Targeting an detargeting adenovirus vectors with polyethylene glycol	10
16	Morizono	Targeting lentiviral vectors for efficient transgene expression in specific cells and tissues	10
17	Lampe	A measles virus with the targeted envelope of another Morbillivirus—Escape from neutralizing antibodies	10
18	Gray	Directed evolution of adeno-associated virus to improve transduction of motor neurons via retrograde transport	10

#	lname	Title	Talk Length
19	Abdelbary	Histone deacetylase inhibitors and VSV—A novel combination tumor targeting therapy	
20	Ebert	Enhancement of transarterial oncolytic VSV therapy by transient reduction of intratumoral blood flow with degradable starch microspheres in multifocal HCC-bearing rats	
21	Alves	Lentiviral-mediated allele-specific silencing of Machado-Joseph disease	
22	Arnett	Heparin-binding properties of AAV6 increase efficiency of muscle transduction in *WT* mice	
23	Basner-Tschakarjan	An in vitro model of AAV vector transduction mimics immune response against transduced human hepatocytes	
24	Brown	Modular rhabdovirus G proteins—A strategy for immune evasion and tissue tropism	
25	cao	Histone deacetylase inhibitors can enhance adenovirus mediate gene expression	
26	Chen	Exploring the use of viral fusogenic membrane glycoproteins to aid viral penetration through the endothelial barrier	
27	Shayakhmetov	The efficiency of post-entry steps of adenovirus infection critically depends on the fiber structure	
28	Ishikawa	Augmentation of neurogenesis by post-ischemic gene transfer of midkine	
29	Fisher	Systemic administration and passive targeting of adenovirus particles	
30	Wilson	Inadvertent gene transfer of the manufacturing process associated viral nucleic acid contaminants and its potential impact on rAAV mediated transgene delivery	
31	Gao	Packaging of rep and cap sequences during production of recombinant AAV and its impact on vector performance	
32	Hojman	Optimized pulses for DNA electrotransfer to muscle tissue significantly reduce damage to the cell membrane	
33	Hui	Pre-existing exposure to AAV in normal donors results in capsid-specific CD8+ T cell responses	
35	Ito	Preparation of very small particles of polyanion-coated DNA complexes for efficient in vivo gene expression	
36	Manley	Anti-apoptotic therapy with a Tat fusion protein protects against excitotoxic insults in vitro and in vivo	
37	Koeberl	Prolonged survival and biochemical correction with an AAV vector in glycogen storage disease type Ia mice and dogs	
38	Li	Low doses of paclitaxel improved antitumor efficacy of adenovirus-mediated gene therapy by enhancing virus distribution in a prostate cancer tumor model	
39	Lopez	Targeting malignant and tumor associated stromal cells as a novel strategy for cancer gene therapy	
40	Marshall	Viral and cellular determinants of lentiviral integration targeting	

41	Morrison	Polymer coated oncolytic adenovirus retargeted with an anti-EGFR antibody reduces toxicity in an in vivo model of ovarian cancer
42	Mougeot	Development of stimuli-responsive nanoparticles for treatment of motor neuron diseases
43	Naik	Developing VSV encoding the sodium iodide symporter and interferon-β gene as a novel vector for the radioiodide imaging and radiovirotherapy of multiple myeloma
44	Noe'	Engineering of rAAV-vectors at promoter and capsid level determinate different NPY gene expression in the hippocampus and mediates its anticonvulsant effects
45	Ohno	APOBEC3 upregulation mediates cancer resistance after successful suicide gene therapy with replication-competent retrovirus vectors
46	Ong	Infection of newly formed blood vessels in mice by integrin targeted measles viruses
47	Power	Carrier cells for oncolytic virus delivery—Investigations of the VSV model
48	Barnes	Transcriptional targeting to the endothelium via the Roundabout-4 receptor promoter
49	Sun	Systematic correction of glycogen storage disease type II by AAV-mediated muscle-specific gene expression
50	Tasciotti	A multi-stage nanodelivery system for the avoidance of biological barriers
51	Xie	Construction of recombinant adenovirus highly expressing human p53 gene lead by Kozak sequence
52	Yang	In utero gene therapy using chitosan-DNA nanoparticles and ΦC31 integrase
53	Ye	HPV E6/E7 regulated differentiation associated genes Hes1/Hes5 and the signal pathway
54	Yoshihara	Improvement of transcriptional efficiency by synthetic polyampholytes

Session 4 VECTOR NEUTRALIZATION

TUESDAY 11/27/2007, 7:30 PM

J. Wilson / J. Bell

#	lname	Title	Talk Length
	Wilson	Title only: Novel DNA viruses to overcome barriers to successful gene therapy	15
55	Bell	Overcoming innate immune responses in the tumor to augement oncolytic virus delivery and spread	15
56	Carlisle	The interaction of adenovirus type 5 with human blood components	10
57	Kreppel	A paradigm for a novel class of glycoviruses—Ad vectors covalently modified with the polysaccharide mannan	10
58	Sinn	Repeated administration of lentiviral vector to mouse nasal epithelia enhanes expression without emergence of blockign mucosal immune responses	10
59	Xu	Clearance of adenovirus by Kupffer cells is mediated by natural antibodies, complement and scavenger receptors	10
60	Roberts	Increased delivery of oncolytic virus PV701 using desensitization and slow intravenous infusion	10

Session 5 PERSISTENCE

WEDNESDAY 11/28/2007, 9:00 AM

K. High / N. Kasahara

#	lname	Title	Talk Length
	High	Title only: AAV transcution of liver and limitations imposed by the human immune response	15
61	Kasahara	Overcoming in vivo barriers to cancer gene therapy with replication-competent retrovirus vectors	15
62	Breous	Both Kupffer cells and hepatic regulatory T cells secrete IL-10 in response to AAV-encoded α-1 antitrypsin	10
63	Chekmasova	High levels of blood cell transgene expression with direct bone marrow injection of SV40-vectors	10
64	Murphy	Incorporation of the SIINFEKL epitope into the VP2 capsid protein of AAV results in minimal MHC-I presentation in the absence of capsid synthesis	10
65	MINGOZZI	Capsid-specific T cell responses in humans upon intramuscular administration of an AAV-1 vector expressing LPL transgene	10
66	Ertl	A pre-clinical animal model to assess the effect of pre-existing immunity to AAV capsid antigens on AAV-mediated gene transfer	10
67	Hirsch	Oligo-Assisted AAV Genome Concatamerization	10
68	Lowenstein	Immune challenges to gene therapy in the brain	10

Session 6 EXTRAVASATION AND TISSUE PENETRATION

WEDNESDAY 11/28/2007, 1:30 PM

K. Bankiewicz / J. Wolff

#	lname	Title	Talk Length
	Bankiewicz	Title only: Use of real time MR imaging for brain delivery of viral vectors	15
69	Wolff	Masked endosomolytic agents—A key feature of synthetic delivery systems for in vivo delivery of siRNA	15
70	Chamberlain	The impact of aging and dystrophy on muscle transduction following IV delivery of AAV	10
71	Ng	Novel strategies to improve liver and lung transduction by helper-dependent adenoviral vectors	10
72	Ota	Cellular carriers for delivery of oncolytic VSVs to sites of myeloma tumor growth	10
73	Weber	Evidence for differential trafficking of single-stranded and double-stranded AAV	10

Special Lecture 5:30 pm Ronald Crystal, Genetic modifications of the central nervous system—From mice to humans 40

Session 7 MANUFACTURING

THURSDAY 11/29/2007, 9:00 AM

M. Federspiel / K. Cornetta

#	lname	Title	Talk Length
75	Cornetta	Challenges to producing clinical grade lentiviral vectors	15
76	Federspiel	The development and optimization of GMP process to produce high titer recombinant measles viruses to phase I/II oncolytic virotherapy clinical trials	10
77	Beecham	Recent advances in overcoming barriers at the manufacturing level	10
78	Schultz	Empty AAV capsids increase transduction of striated muscle	10
79	Gao	Transgene immunity, a potential barrier to AAV mediated and liver directed gene therapy—Case studies in the nonhuman primate model	10