

Program

Oligonucleotide Delivery: *Biology, Engineering and Development*

October 7-11, 2012

Schloss Hernstein
Hernstein, Austria

Conference Chairs:

Laura Sepp-Lorenzino
Merck & Co., Inc., USA

Steve Dowdy
UCSD School of Medicine, USA



Engineering Conferences International

32 Broadway, Suite 314 - New York, NY 10004, USA

Phone: 1 - 212 - 514 - 6760, Fax: 1 - 212 - 514 - 6030

www.engconfintl.org – info@engconfintl.org

Schloss Hernstein
Berndorfer Straße 32
A-2560 Hernstein
Austria
Tel: +43 (0)2633 47 251

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Sunday, October 7, 2012

16:00 – 18:00	Conference Check-in (Schloss Hernstein Lobby)
17:00 – 17:30	Welcome and General Announcements Conference Chairs ECI Technical Liaison
17:30 – 18:30	<u>Plenary Talk</u> Design of lipid nanoparticle systems for the systemic delivery of siRNA Pieter Cullis, University of British Columbia, Canada
18:30 – 19:30	Opening Reception (historic area of castle)
19:30 – 21:00	Dinner (Schloss Restaurant)
21:00 – 22:00	Social period (bar)

NOTES

- *Technical sessions will be held in Hofsuite 1-2*
- *Coffee breaks will be outside the technical session room*
- *Meals will be buffet with a vegetarian option*
- *Audiotaping, videotaping and photography of presentations are prohibited.*
- *Speakers – Please have your presentation loaded onto the conference computer prior to the session start (preferably the day before).*
- *Speakers – Please leave at least 5 minutes for questions and discussion.*
- *Please do not smoke at any conference functions.*
- *Turn your mobile phones to vibrate or off during technical sessions.*

Monday, October 8, 2012

- 07:30 – 08:30 Breakfast
- 08:30 – 12:00 **Session I: Delivery Strategies #1**
Session Chair: Marino Zerial, Max Plank Institute of Molecular Cell Biology and Genetics MPI-CBG, Germany
- 08:30 – 08:55 **Sequence-defined polymers as dynamic carriers for targeted nucleic acid delivery**
Ernst Wagner, Ludwig-Maximilian University, Germany
- 08:55 – 09:20 **Designed ankyrin repeat proteins for tumor-specific siRNA delivery**
Johannes Winkler, University of Vienna, Austria
- 09:20 – 09:45 **Improved intratumoral delivery of PEG-coated siRNA-lipoplexes by combination with metronomic S-1 dosing in a murine solid tumor model**
Tatsuhiko Ishida, University of Tokushima, Japan
- 09:45 – 10:15 Coffee Break
- 10:15 – 10:35 **Quantitative light and electron microscopy analysis of lipid nanoparticle-mediated siRNA delivery and intracellular trafficking**
Marino Zerial, Max Plank Institute of Molecular Cell Biology and Genetics (MPI-CBG), Germany
- 10:35 – 11:00 **Combined targeted chemo and gene therapy for tumor suppression and prevention of metastases**
Tamara Minko, Rutgers, The State University of New Jersey, USA
- 11:00 – 11:25 **Direct selection of internalizing RNAs**
Matthew Levy, Albert Einstein College of Medicine, USA
- 11:25 – 11:50 **Targeting tumor angiogenesis using RNA interference**
Anil Sood, University of Texas, USA
- 11:50 – 12:05 **A strategy for efficient, targeted siRNA delivery to submucosal macrophages**
Randall Mrsny, University of Bath, United Kingdom
- 12:05 – 14:00 Lunch
- 14:00 – 18:00 **Session II: Delivery Strategies #2**
Session Chair: Ernest Wagner, Ludwig-Maximilian University, Germany
- 14:00 – 14:25 **Systemic delivery of RNAi therapeutics using conjugates**
Mano Manoharan, Alnylam Pharmaceuticals, USA
- 14:25 – 14:50 **CNS and siRNA therapeutics: targeting aminergic neurons for silencing mood disorders-related genes**
Andres Montefeltro, nLife Therapeutics, S.L., Spain
- 14:50 – 15:15 **Novel therapeutic nanoparticles for in vivo delivery of low dose siRNA in liver cells**
Xavier de Mollerat Du Jeu, Life Technologies, USA

Monday, October 8, 2012 (continued)

- 15:15 – 15:40 **Anti-miRNA and splice-switching oligonucleotides with a novel non-base chemical modifier**
Mark Behlke, Integrated DNA Technologies, USA
- 15:40 – 16:05 **Non-viral delivery of siRNA in vivo**
Jorgen Kjems, Aarhus University, Denmark
- 16:05 – 16:35 Coffee Break
- 16:35 – 16:55 **Carbohydrates as ligands for delivery**
Peter Seeberger, Max-Planck Institute, Germany
- 16:55 – 17:20 **Targeted exosomes for systemic delivery of siRNA**
Samir El Andaloussi, Karolinska Institute, Sweden
- 17:20 – 17:35 **TBD**
- 18:00 – 19:15 Dinner
- 19:15 – 20:45 Wine Tasting (historic area of castle)
- 20:45 – 21:00 **Posters** with Social Hour

Tuesday, October 9, 2012

- 07:30 – 08:30 Breakfast
- 08:30 – 12:00 **Session III: Delivery Strategies #3**
Session Chair: Gilles Divita, Centre National De La Recherche Scientifique, France
- 08:30 – 08:55 **Enhancing endosomal escape to improve the pharmacological effects of oligonucleotides**
Rudy Juliano, University of North Carolina, USA
- 08:55 – 09:20 **Development of endosomolytic peptides: Finding a way out of endosomes for the delivery of macromolecules into live cells**
Jean Phillipe Pellois, Texas A&M University, USA
- 09:20 – 09:45 **Polymerizable lipid-based multifunctional siRNA delivery systems**
Zheng-Rong Lu, Case Western Reserve University, USA
- 09:45 – 10:15 **Coffee Break**
- 10:15 – 10:35 **Polymeric nanoparticles as delivery platform for small RNA molecules (siRNAs, miRNAs) *in vivo*: development of novel therapeutic strategies based on gene silencing**
Achim Aigner, University of Leipzig, Germany
- 10:35 – 11:00 **Delivery of siRNA to microvascular endothelial cells by lipid-based carriers**
Jan Kamps, UMCG, The Netherlands
- 11:00 – 11:25 **Conformationally-constrained kinked peptides potently mediate escape of cargo from early endosomes**
Blake Peterson, University of Kansas, USA
- 11:25 – 11:50 **Design of functional polymeric nanomaterials for nucleic acid delivery**
Kanjiro Miyata, University of Tokyo, Japan
- 11:50 – 12:05 **Therapeutic antagonists of microRNAs deplete leukemia-initiating cell activity**
H. Leighton Grimes, Cincinnati Childrens Hospital, USA
- 12:05 – 14:00 **Lunch**
- 14:00 – 18:00 **Session IV: Delivery Strategies #4**
Session Chair: Anil Sood, University of Texas, USA
- 14:00 – 14:25 **Targeted delivery of small interfering RNA with RGD-decorated polycation liposomes for cancer therapy**
Tomohiro Asai, University of Shizuoka, USA
- 14:25 – 14:50 **Multi-stage vectors for siRNA cancer therapeutics**
Haifa Shen, Methodist Hospital Research Institute, USA
- 14:50 – 15:15 **RNAi –based nanomedicine: Directing the immune response?**
Dan Peer, Tel Aviv University, Israel

Tuesday, October 9, 2012 (continued)

- 15:15 – 15:40 **Cell-penetrating peptides in gene delivery**
Ulo Langel, Stockholm University, Sweden
- 15:40 – 16:05 **Peptide-based nanoparticles for in vivo targeted delivery of siRNA**
Gilles Divita, Centre National De La Recherche Scientifique, France
- 16:05 – 16:35 Coffee break
- 16:35 – 16:55 **Novel siRNA-amino acid conjugates exhibit improvements in cellular uptake and reduce DRR expression in tumor cells**
Masad Damha, McGill University, Canada
- 16:55 – 17:20 **Induction of RNAi responses by bioreversible phosphotriester siRNNs**
Steve Dowdy, UCSD School of Medicine, USA
- 17:20 – 17:35 **RNA aptamers as tools for specific cancer cells targeting and delivery**
Vittorio De Franciscis, Istituto di Endocrinologia ed Oncologia Sperimentale CNR, Italy
- 18:00 – 19:15 Dinner
- 20:00 – 22:00 **Posters** with Social Hour

Wednesday, October 10, 2012

- 07:30 – 08:30 Breakfast
- 08:30 – 12:00 **Session V: Therapeutic oligonucleotide programs**
Session Chair: Pieter Cullis, University of British Columbia, Canada
- 08:30 – 08:55 **Discrete cellular uptake and structure activity determinants of RNA**
Troels Koch, Santaris Pharma A/S, Denmark
- 08:55 – 09:20 **Ophthalmic application of siRNA, from conception to phase 2**
Roberto Weinmann, Pharmamar USA and Sylentis, USA
- 08:20 – 09:45 **ATU027: Development of an RNAi therapeutic in oncology**
Ansgar Santel, Silence Therapeutics AG, Germany
- 09:45 – 10:15 Coffee Break
- 10:15 – 10:35 **RNAi-based treatment for pancreatic cancer: Pre-clinical and clinical results**
Amotz Shemi, Silenseed LTD, Israel
- 10:35 – 11:00 **Systemic delivery of RNAi therapeutics using LNPs**
Mano Manoharan, Alnylam Pharmaceuticals, USA
- 11:00 – 11:25 **Challenges in discovery and development of nucleic acid therapeutics**
Sabine Scheidler, Sanofi-Aventis Deutschland GmbH, Germany
- 11:25 – 11:50 **Functionalized, biodegradable hydrogels for control over sustained and localized siRNA delivery to incorporated and surrounding cells**
Eben Alsberg, Case Western Reserve University, USA
- 12:10 Meet in lobby and pick up boxed lunch
- 12:15 – 18:00 Excursion to Vienna – bus leaves promptly at 12:15.
Time to spend the afternoon discovering this beautiful city on your own with a provided map or to see the city on a Hop-On, Hop-Off bus tour. (Bus tour paid directly to company.)
Conference bus leaves Vienna at 17:15 promptly.
- 18:30 – 19:15 **Plenary Talk 2**
Trials and tribulations of big pharma RNAi
Hans-Peter Vornlocher, Axolabs GmbH, Germany
- 19:15 – 21:30 Conference banquet

Thursday, October 11, 2012

07:30 – 08:30	Breakfast
08:30 – 12:00	<u>Session VI: PK, biodistribution, safety</u> Session Chair: Rudy Juliano, University of North Carolina, USA
08:30 – 08:55	Delivery of cargoes to dendritic cells <i>in situ</i> Debbie Palliser, Albert Einstein College of Medicine, USA
08:55 – 09:20	TBA
09:20 – 09:45	Delivery of siRNAs to lung and vaginal tract for the treatment of cancer and viral disease Nigel McMillan, Griffith University, Australia
09:45 – 10:15	Coffee Break
10:15 – 10:35	TBA Sterghios Moschos, University of Westminster, United Kingdom
10:35 – 11:00	Logic embedded vectors for intracellular targeting Rita Serda, Methodist Hospital Research Institute, USA
11:00 – 11:25	TBA Gabriel Lopez-Berestein, University of Texas, USA
11:25 – 11:50	Oligo delivery gap analysis: Open discussion Laura Sepp-Lorenzino, Merck, USA
11:50 – 12:00	Closing Remarks: Laura Sepp-Lorenzino, Merck Steve Dowdy, UCSD
12:00	Lunch and departures

Oligonucleotide Delivery: Biology, Engineering and Development

Poster List

- 1. Development of aptamer-chimera therapeutics against cell surface receptors expressed by leukemia and adenocarcinomas**
Elizabeth D. Pratico, Duke University Medical Center, USA
- 2. Targeted liposomes for siRNA delivery to Dendritic Cells in vivo**
Joseph Katakowski, Albert Einstein College of Medicine, USA
- 3. Ugimers - Expanding the therapeutic gene silencing space with innovative modular chemistry**
Jürgen Soutschek, Ugichem GmbH, Austria
- 4. Recombinant Dynein light chain Lc8 as non viral delivery vector for gene therapy – exploring the microtubules transport network to enhance the traffic of exogenous DNA in the cytosol.**
Marcelo Augusto Szymanski de Toledo, Universidade Estadual de Campinas, Brazil
- 5. Novel Cell-Penetrating Oligomers (CPOs) for highly efficient cargo delivery to mammalian cells**
Moneesha Fernandes, National Chemical Laboratory, India
- 6. Low-molecular-weight supramolecular hydrogel of riboflavin bolaamphiphile for VEGF-siRNA delivery**
Sachin Prakash Patil, Pohang University of Science and Technology, Korea
- 7. Targeted siRNA delivery via aptamer-conjugated liposomes**
Samantha E. Wilner, Albert Einstein College of Medicine, USA
- 8. LNA- oligonucleotides - From class effects to unique sequence-dependent properties**
Susanne Kammler, Santaris Pharma A/S, Denmark
- 9. A new non-base chemical modifier “ZEN” enhances efficacy of splice-switching oligonucleotides**
Suzan M Hammond, University of Oxford, United Kingdom
- 10. Syndecans as targeted drug delivery vehicles**
Tamas Letoha, Pharmacoidea Ltd., Hungary
- 11. Synthesis of phosphoramides polyethyleneimine derivatives as potential vehicles for targeting antitumor compound to cancer cells and molecular probes for ³¹P magnetic resonance spectroscopy in vivo**
Tatyana S. Godovikova, Institute of Chemical Biology and Fundamental Medicine SB RAS, Novosibirsk, Russia
- 12. Nanobiocomposites SIO2-azidothymidine: design and properties**
Vladimir Silnikov, Institute of Chemical Biology and Fundamental Medicine SB RAS, Novosibirsk, Russia
- 13. Antiangiogenic therapy by delivery of MIR-92A to angiogenic endothelial cells**
Hidenori Ando, University of Shizuoka, Japan

14. **Enhanced muscle uptake and efficacy of antisense oligonucleotide ligand conjugates in mdx mice**
M.B. Aguilera, Prosensa Therapeutics, the Netherlands
15. **Mechanism of cellular uptake of self-assembling CADY/siRNA nanoparticles**
Sebastien Deshayes, CRBM-CNRS-UMR5237, France
16. **Characterization of self-assembling CADY/siRNA nanoparticles**
Karidia Konate, CRBM-CNRS-UMR5237, France