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
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www.engconfintl.org – info@engconfintl.org
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  **Plenary Talk**
               *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**  
Tatsuhiro 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
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<tr>
<th>Time</th>
<th>Event</th>
<th>Speaker/Institution</th>
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<tbody>
<tr>
<td>15:15 – 15:40</td>
<td><strong>Cell-penetrating peptides in gene delivery</strong></td>
<td>Ulo Langel, Stockholm University, Sweden</td>
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<tr>
<td>15:40 – 16:05</td>
<td><strong>Peptide-based nanoparticles for in vivo targeted delivery of siRNA</strong></td>
<td>Gilles Divita, Centre National De La Recherche Scientifique, France</td>
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<td>16:05 – 16:35</td>
<td>Coffee break</td>
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<td>16:35 – 16:55</td>
<td><strong>Novel siRNA-amino acid conjugates exhibit improvements in cellular uptake and reduce DRR expression in tumor cells</strong></td>
<td>Masad Damha, McGill University, Canada</td>
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<td>16:55 – 17:20</td>
<td><strong>Induction of RNAi responses by bioreversible phosphotriester siRNAs</strong></td>
<td>Steve Dowdy, UCSD School of Medicine, USA</td>
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<td>17:20 – 17:35</td>
<td><strong>RNA aptamers as tools for specific cancer cells targeting and delivery</strong></td>
<td>Vittorio De Franciscis, Istituto di Endocrinologia ed Oncologia Sperimentale CNR, Italy</td>
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<td>18:00 – 19:15</td>
<td>Dinner</td>
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<td><strong>Posters with Social Hour</strong></td>
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**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  **Oligo delivery gap analysis: Open discussion**  
   Laura Sepp-Lorenzino, Merck, 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  
**Session VI: PK, biodistribution, safety**
Session Chair: Rudy Juliano, University of North Carolina, USA

08:30 – 08:55  
**Delivery of cargoes to dendritic cells in situ**
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  
**Functionalized, biodegradable hydrogels for control over sustained and localized siRNA delivery to incorporated and surrounding cells**
Eben Alsberg, Case Western Reserve University, USA

11:25 – 11:50  
**TBA**
Gabriel Lopez-Berestein, University of Texas, USA

11:50 – 12:00  
Closing Remarks:
Laura Sepp-Lorenzino, Merck
Steve Dowdy, UCSD

12:00  
Lunch and departures
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 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 $^{31}$P magnetic resonance spectroscopy in vivo
    Tatyana S. Godovikova, Institute of Chemical Biology and Fundamental Madicine SB RAS, Novosibirsk, Russia

12. Nanobiocomposites SIO2-azidothymidine: design and properties
    Vladimir Silnikov, Institute of Chemical Biology and Fundamental Madicine 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

   Karidia Konate, CRBM-CNRS-UMR5237, France