

WORKSHOP TITLE

Emerging Trends in Gene/Cell-based Therapeutics and Nanocarriers for Drug Delivery

WORKSHOP CHAIRS

Ram I. Mahato (University of Nebraska Medical Center), Weiyue Lu (Fudan University) and Chalet Tan (Mercer University)

DATE & PLACE

April 24-25, 2014 at Fudan University, Shanghai, China

CO-SPONSORS

Chinese Association of Pharmaceutical Sciences
American Chinese Pharmaceutical Association
AAPS Nanotechnology Focus Group

WORKSHOP OUTLINES

Advancements in smart polymers and more efficient carriers systems have propelled RNAi therapeutics closer to the clinic. They have also posed many unanswered questions pertaining to the relative roles and therapeutic potentials of small-interfering RNA (siRNA), short hairpin RNA (shRNA), microRNA (miRNA), antisense oligonucleotides (ODNs), plasmid DNA (pDNA) and RNA/DNA aptamers. Identification of the most effective delivering and targeting strategies of these nucleic acids remains a major hurdle in turning them into therapeutics. Moreover, the emergence of stem cell-based therapies (mesenchymal stem cells, embryonic stem cells and induced pluripotent stem cells) has added a new dimension to medical interventions. Furthermore, nanotechnology has brought us much closer than ever in realizing the long cherished dream of “magic bullets” by enabling spatio-temporal delivery of drugs in a manner, which is most appropriate for treating the underlying pathophysiology of severe diseases. This workshop will assemble the leading experts to address the latest advances in nucleic acid/cell/drug delivery, from basic research to pre-clinical testing to clinical applications. The workshop will provide a multidisciplinary forum and unique opportunity for pioneers as well as novice in the field to expand their knowledge and awareness on the emerging trends in nanotechnology, and discuss some of the most critical concerns related to the design, evaluation and regulatory hurdles hindering the progress in nanomedicine. Workshop attendees will in particular gain insights into the following:

- 1. Physicochemical and pharmaceutical considerations for successful gene therapy**
Ram I. Mahato, University of Nebraska Medical Center, USA
- 2. Recent advances in nucleic acid-based therapeutics**
Frank Szoka, University of California San Francisco, USA

- 3. Opportunities and challenges for RNAi technologies**
Mark Davis, California Institute of Technology, USA
- 4. miRNAs as targets for cancer treatment: identification, validation and delivery**
Frank J. Slack, Yale University, USA
- 5. Design of Biomaterials for Small Molecules, Proteins and Nucleic Acid Drugs**
Sung Wan Kim, University of Utah, USA
- 6. Concurrent Delivery of microRNA and Small-molecule Drugs**
Chalet Tan, Mercer University, USA
- 7. Functional Biomaterials for Tissue Engineering and Regenerative Medicines**
Dong-an Wang, Nanyang University, Singapore
- 8. Viral vectors: design, advantages and challenges**
Chae-Yuk Yoon, Hanyang University, Seoul, Korea
- 9. Recent advances in cell-based therapeutics**
Teruo Okano, Tokyo Women's Medical University, Japan
- 10. Mesenchymal stem cells in oncology and regenerative medicine**
Kam Leong, Duke University, USA
- 11. siRNA and miRNA-associated in vivo toxicity: safety and regulatory consideration**
Lawrence Y, Food and Drug Administration, USA