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SMi presents their 9th annual conference on

RNA Therapeutics

Advancing, Developing And Commercialising Novel
RNA Therapeutics and their Delivery Systems

HOLIDAY INN KENSINGTON FORUM, LONDON, UK

CONFERENCE:
21ST – 22ND

FEB
2018

FEATURED HIGHLIGHTS IN 2018:

- Hear from **MiNA Therapeutics** about their **new pre-clinical data** supporting the on-target mechanism of action of their **pioneering new drug candidate MTL-CEBPA**
- Learn how **CureVac's** sophisticated technologies and approaches have helped **overcome limitations** such as the **instability of single-stranded RNA**
- Discover **STORM Therapeutics'** innovative approach: **Harnessing the power of RNA epigenetics to develop first-in-class drugs in oncology**
- Expand your knowledge of **patenting RNA Therapeutics**, with James Ogle, a European and UK Chartered Patent Attorney with **particular expertise in RNA and other nucleic acid-based technologies**
- Examine the **latest developments in delivery systems** for RNA Therapeutics, including **Exosomes, Nanocomplexes and Platform Delivery Approaches**

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CHAIRS FOR 2018:



Nagy Habib, Head of HPB Surgery, **Imperial College London** and Co-Founder, **MiNA Therapeutics**



Shalini Andersson, Senior Director Drug Metabolism & Pharmacokinetics, **AstraZeneca R&D**

KEY SPEAKERS INCLUDE:



Ekkehard Leberer, Senior Director, **Sanofi**



Jeffrey Ulmer, Head Preclinical R&D, **GSK**



James Hamilton, Vice President, Head of Clinical Development, **Arrowhead Pharmaceuticals**



Mariola Fotin-Mleczek, Chief Scientific Officer, **CureVac**



Bob D. Brown, Chief Scientific Officer and Senior Vice President, Research, **Dicerna Pharmaceuticals**



Oliver Rausch, VP Research & Development, **Storm Therapeutics**



Kirsty Wydenbach, Senior Medical Assessor, Deputy Unit Manager, Clinical Trials Unit, **MHRA**



www.therapeutics-rna.com

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8.30 **Registration & Coffee**

9.00 **Chair's Opening Remarks**

Nagy Habib, Head of HPB Surgery, **Imperial College London** and Co-Founder, **MiNA Therapeutics**

USING RNA TO TREAT DISEASES

OPENING KEYNOTE ADDRESS

9.10 **Engineering a Novel Antibiotic Against a Unique RNA Target that Prevents Resistance**

- Target selection unique to Gram positive pathogens
- Screening tools for putative small molecule, RNA binding antibiotics against Gram positive pathogens and their biofilms
- Target prevents emergence of resistance
- Low Cytotoxicity and Toxicity

Paul Agris, Founding Director, **The RNA Institute, SUNY University at Albany**

KEYNOTE ADDRESS

9.50 **Utilising Small Activating RNA (saRNA) for Therapeutic Opportunities**

- Harnessing the innate mechanism of gene activation
- Evaluating how saRNA can be used to restore the expression of CCAAT/enhancer binding protein alpha (C/EBP-α)
- Achieving proof of concept: Clinical applications and therapeutic opportunities this offers
- Lessons learned during Phase I Clinical Study of MTL-CEBPA in Patients with Liver Cancer

Nagy Habib, Head of HPB Surgery, **Imperial College London** and Co-Founder, **MiNA Therapeutics**

10.30 **Morning Coffee**

11.00 **RNA therapeutics for patients with cystic fibrosis: QR-010 and beyond**

- Opportunities for RNA therapeutics in patients with cystic fibrosis
- Evidence for QR-010 as a meaningful RNA therapeutic for patients with cystic fibrosis due to the F508del mutation
- Potential RNA therapeutic approaches for patients with cystic fibrosis due to other mutations

Noreen Roth Henig, Chief Medical Officer, **ProQR Therapeutics**

KEYNOTE ADDRESS

11.40 **MicroRNA Therapeutics: Opportunities and Challenges for Targeting the Pathways of Human Disease**

- MicroRNAs regulate by the mechanism of RNA interference (RNAi) biochemical pathways involved in human diseases
- MicroRNA-21 has been implicated in multiple organs with fibrosis and cancer
- The presentation will summarize opportunities and challenges of developing microRNA based oligonucleotide therapeutics and illustrate the generation of an anti-fibrotic oligonucleotide drug targeting microRNA-21

Ekkehard Leberer, Senior Director, **Sanofi**

12.20 **miRNases: Novel peptide-oligonucleotide bioconjugates that silence miR-21 in lymphosarcoma cells**

- The key challenges in the traditional DDD process of small molecule drugs
- Some emerging opportunities in attempt to overcome the key issues
- New avenues for development of novel therapeutic interventions against cancer
- Development of 'intelligent' anti-cancer therapeutics against oncogenic microRNAs

Elena Bichenkova, Senior Lecturer in Medicinal Chemistry, **Manchester University**

13.00 **Networking Lunch**

14.10 **Developing mRNA therapeutics for Skin Diseases**

- Skin offers various opportunities with regard to development of mRNA-based therapeutics: diseases with validated molecular targets/attractive markets and direct access facilitating quantification of mRNA expression/clinical activity
- mRNA is a new drug format capable of exceeding existing protein-based therapeutics.
- Developing proprietary IVT-mRNAs addressing validated targets for specific skin conditions
- Systematically modifying specific IVT-mRNAs and testing the most interesting ones in skin explant systems varying formulation and delivery

Markus Mandler, Chief Scientific Officer, **Accanis Biotech F&E GmbH & Co KG**

NOVEL APPROACHES AND EMERGING TECHNOLOGIES

KEYNOTE ADDRESS

14.50 **Self-amplifying mRNA vaccines**

- Evaluating the new vaccine technology known as SAM (self-amplifying mRNA)
- Utilising mRNA research alongside recombinant adenoviral vector technology
- Case study: Lessons learned when attempting to develop a Zika vaccine

Jeffrey Ulmer, Head Preclinical R&D, **GSK**

15.30 **Afternoon Tea**

16.00 **Harnessing the power of RNA Epigenetics - targeting RNA modifying enzymes for the treatment of cancer**

- Over 150 different chemical modifications have been identified on RNA molecules.
- The role of these modifications in the maturation and regulation of coding and non-coding RNA is becoming increasingly prominent.
- Utilising the research of world-leading founders Professors Kouzarides and Miska in the field of RNA epigenetics to pioneer the development of small molecule inhibitors of previously untargeted RNA-modifying enzymes (RMEs) to interfere with key RNA pathways and mechanisms

Oliver Rausch, VP Research & Development, **Storm Therapeutics**

16.40 **RNAoptimizer - From mRNA to product**

- CureVac's RNAoptimizer provides optimal mRNA for each target indication
- The RNAoptimizer includes sequence optimization, formulation & delivery adjustment and protein design improvement
- RNAoptimizer is developed as robotic assisted process

Mariola Fotin-Mleczek, Chief Scientific Officer, **CureVac GmbH**

17.20 **Chair's Closing Remarks and Close of Day One**

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Phion Therapeutics Ltd has developed a peptide nanoparticle for drug delivery that can deliver nucleic acids and other anionic compounds

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8.30 Registration & Coffee

9.00 Chair's Opening Remarks

Shalini Andersson, Senior Director Drug Metabolism & Pharmacokinetics, **AstraZeneca R&D**

CONSIDERATIONS AND REGULATIONS

OPENING ADDRESS

9.10 Gaining Regulatory Approval and Mitigating Risks during Clinical Trials

- Expectations of a regulator and how to report your results
- Overcoming common governing pitfalls
- Updates of new and upcoming regulations and how you may be affected

Kirsty Wydenbach, Senior Medical Assessor, Deputy Unit Manager, Clinical Trials Unit, **MHRA**

9.50 Patenting RNA Therapeutics

- Drafting a successful new patent application
- Advice regarding oppositions and appeals before the European Patent Office
- Avoiding risks associated with patents, including patent infringement and patent transactions
- Moving beyond compound protection, which types of follow-up protection should be considered by innovators

James M. Ogle, Partner, European and UK Patent Attorney, **Hoffmann Eitle**

10.30 Morning Coffee

ENHANCING DELIVERY SYSTEMS

11.00 Local and Systemic Oligonucleotide Delivery Using RALA Peptide Nanoparticles

- High Transfection Efficiency with Low Associated Toxicity
- Tunable Systemic In Vivo Biodistribution
- Ability to Concentrate into Tumours/Cancer Cells
- Simple, Easy to Use Formulation and Scale Up

Helen McCarthy, Chair of Nanomedicine, **Queen's University Belfast's, School of Pharmacy**

KEYNOTE ADDRESS

11.40 Advances in Targeted Delivery of Nucleotides Beyond the Liver

- Potential targeting approaches for delivery to tissues and cell types beyond the liver, including the cardiovascular, metabolic and renal systems
- Overcoming challenges, including insufficient pharmacokinetic properties and poor cellular uptake
- Evaluation of the drugs in the pipeline for AstraZeneca

Shalini Andersson, Senior Director Drug Metabolism & Pharmacokinetics, **AstraZeneca R&D**

12.20 Viromer preclinical validation

- The Viromer technology
- Optimization both for Chemistry and Formulation
- Application in Preclinical Models

Steffen Panzner, Managing Director, **Lipocalyx**

13.00 Networking Lunch

14.10 Utilising an alternative siRNA delivery approaches to target liver hepatocytes

- Advantages of using GalXC RNAi platform delivery approach over a traditional Lipid Nanoparticle (LNP) Delivery System
- Chemistry and challenges of this platform
- Advantages of using Dicer Substrate short-interfering RNAs (DsiRNAs), for targeted therapies
- Case study: Dicerna's drug pipeline and pre-clinical trials, which utilize the alternative delivery approach to treat disease

Bob D. Brown, Chief Scientific Officer, SVP Research & Development, **Dicerna Pharmaceuticals**

14.50 The Evolution of RNAi Delivery at Arrowhead

- Older RNAi delivery technologies
- Dynamic Polyconjugates: Pre-clinical and Clinical Experience
- Arrowhead Single Molecule RNAi Conjugates

James Hamilton, Vice President, Head of Clinical Development, **Arrowhead Pharmaceuticals, Inc.**

15.30 Afternoon Tea

16.00 Unlocking the potential of nucleic acid therapeutics using Spherical Nucleic Acids

- Spherical Nucleic Acid (SNA™) are nanoscale, spherical arrangements of densely packed and radially oriented nucleic acids
- Localization to endosome permits large numbers of SNA's to be delivered with low toxicity
- By activating innate immune cells, SNA's enhance the function and secretion of cytokines
- AST-008 is a TLR9 agonist oligonucleotide in SNA format which demonstrates greater delivery to primary cells and more potent TLR9 activation in cell-based assays:
 - AST-008 has been evaluated in numerous solid and liquid tumor models, including breast, colon, bladder, melanoma and lymphoma
 - Combining AST-008 with a checkpoint inhibitor (PD-1) and IDO inhibitors results in a profound impact on tumor growth and survival
 - AST-008 clinical trial progress will be discussed

David Giljohann, CEO, **Excure Inc.**

16.40 Exploiting Exosomes for therapeutic and diagnostic applications

- Examining the translational applications of exosome research, including biomarker discovery and drug delivery
- Consideration and evaluation of the important advantages compared to other nanoparticulate drug delivery systems
- Overcoming potential problems with component characterization and immune reactions

Samir EL Andaloussi, Assistant Professor, **Karolinska Institutet** and Research Fellow, **University of Oxford**

17.20 Delivery of a MYCN siRNA Therapy to Neuroblastoma by a Targeted Nanocomplex

- Neuroblastoma is a tumour of nerves with primary tumours usually located in the abdomen
- Neuroblastoma is the most common solid tumour in childhood with worst prognosis cases involving amplification of the MYCN proto-oncogene
- Silencing of MYCN induces differentiation, apoptosis leading to tumour eradication or slow growth
- Integrin-targeted, lipid-peptide nanocomplexes enable systemic delivery to neuroblastoma tumours specifically, avoiding uptake by liver and other organs

Stephen Hart, Professor in Molecular Genetics, **University College London**

18.00 Chair's Closing Remarks and Close of Day Two

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