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SMi Presents the 10th Annual Conference on...

RNA Therapeutics

Advances in delivery technologies and applications
of RNA-based drugs for modulation of gene, protein
expression, and genome editing

COPTHORNE TARA HOTEL, LONDON, UK

CONFERENCE:
20TH-21ST

FEB
2019

CHAIRS FOR 2019:

- **Ekkehard Leberer**, Senior Director, Alliance Management, **Sanofi**
- **Shalini Andersson**, Senior Director and Head of New Modalities, **AstraZeneca**

GUEST SPEAKER:

- **Paul Agris**, Founder and Director, **The RNA Institute**

KEY SPEAKERS:

- **Carsten Rudolf**, Chief Executive Officer, **Ethris GmbH**
- **Marian Gindy**, Executive Director, Pharmaceutical Sciences, Merck Research Labs, **Merck & Co, Inc.**
- **David Giljohan**, CEO, **Exicure**
- **Ingmar Hoerr**, Founder and CEO, **CureVac AG**
- **David Blakey**, Chief Scientific Officer, **MIANA Therapeutics**
- **André Gerber**, Professor of RNA Biology, **University of Surrey**
- **Heinrich Haas**, Vice President Drug Delivery, **BioNTech RNA Pharmaceuticals**
- **Markus Mandler**, Chief Scientific Officer, **Accanis Biotech**
- **Troels Koch**, Vice President & Head of Research, RNA Therapeutics, **Roche**
- **Hans Kistemaker**, Associate Director, **ProQR**

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2019 FEATURED HIGHLIGHTS:

- **Discover** the clinical progress of Spherical Nucleic Acids at **Exicure**
- **Understand Sanofi's** strategies to deliver therapeutic oligonucleotides across biobarriers
- **Learn** from **ProQR** how to plan for oligonucleotide supply from personalized medicine to large scale markets
- **Explore** the development of small activating RNA – from bench to bedside with **MIANA Therapeutics**
- **Gain insight** into the discovery and development of self-amplifying mRNA vaccines at **GSK**



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8.30 Registration & Coffee

9.00 Chair's Opening Remarks

Ekkehard Leberer, Senior Director, Alliance Management, Sanofi-Aventis

RECENT ADVANCEMENTS IN OLIGONUCLEOTIDE THERAPEUTICS' R&D

OPENING ADDRESS

9.10 Spherical Nucleic Acids: Clinical progress

- Localization to endosome permits large numbers of SNA's to be delivered with low toxicity
- SNAs can be used to knockdown genes of interest, or activate immune pathways productively
 1. AST-008 TLR9 agonist clinical trial progress will be discussed
 2. XCUR-17, Topical antisense in psoriasis will be discussed

David Giljohan, CEO, **Excicure**

9.50 LNA therapeutics: Recent developments & discovery concepts

- Locked nucleic acid (LNA) has over the last fifteen years become a leading RNA therapeutic modality and is today one important reason for the high interest in oligonucleotide medicines
- The combination of LNA nucleosides and novel phosphorothioate chemistries enables bespoke tailoring of LNA drug designs
- Such new drug designs and new discovery concepts have improved RNA therapeutic drug discovery significantly
- The presentation will review the recent developments, and also show how this modality is constantly improved by novel nucleotide modifications, designs, modelling and delivery technologies

Troels Koch, Vice President & Head of Research, RNA Therapeutics, **Roche**

10.30 Morning Coffee

11.00 Case study: Review of the engineering and application of 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, Founder and Director, **The RNA Institute**

11.40 SPOTLIGHT: Self-amplifying mRNA vaccines

- Induction of broad and potent immune responses
- Synthetic production methods
- Amenable to rapid response against newly emerging infectious diseases
- Potential disruptive technology to streamline vaccine discovery and development

Jeffrey Ulmer, Head, Preclinical Research and Development US, **GlaxoSmithKline**



12.20 Networking Lunch

NOVEL mRNA TECHNOLOGY

1.20 Profiling the rearrangement of RNA-binding proteins on cancer-related mRNAs identifies modulators of drug sensitivity

- Cisplatin induces post-transcriptional regulation of a cancer-related mRNA
- New biochemical approach to capture particular mRNAs and bound proteins
- RNA-binding proteins modulate drug sensitivity of cancer cells

André Gerber, Professor of RNA Biology, **University of Surrey**

2.00 Innovative mRNA vaccines against flu and malaria

- Introducing CureVac's RNAActive® prophylactic vaccine technology
- Development of mRNA-based vaccines designed to prevent influenza and malaria infection
- mRNA platform enables the cost-effective and fast manufacturing of vaccines to prevent diseases
- Advantages include: flexible applications, rapid production and potential to address several global vaccine challenges

Ingmar Hoerr, Founder and CEO, **CureVac AG**

2.40 Afternoon Tea

3.10 Development of the novel GalXC™ RNAi therapeutics for the treatment of chronic liver diseases

- GalXC technology platform uses RNAi to inhibit the expression of disease-causing genes by destroying the messenger RNAs (mRNAs) of selected genes
- Potential to treat diseases by silencing previously inaccessible drug targets
- Development of novel RNAi therapies for non-alcoholic steatohepatitis and other chronic liver diseases

Bob Brown, CSO and SVP Res & Dev, **Dicerna Pharmaceuticals**

3.50 Towards 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.
- ACCANIS develops proprietary IVT-mRNAs addressing validated targets for specific skin conditions
- We systematically modified specific IVT-mRNAs and tested the most interesting ones in ex vivo and in vivo skin model systems varying formulation and delivery

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

4.30 Chair's Closing Remarks and Close of Day One

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8.30 Registration & Coffee

9.00 Chair's Opening Remarks

Shalini Andersson, Senior Director and Head of New Modalities, AstraZeneca

DEVELOPMENTS IN DELIVERY MECHANISMS FOR RNA THERAPEUTICS

OPENING ADDRESS

9.10 Development of formulations for delivery of RNA and small molecules

- Technical challenges of systemic delivery of vaccine antigens into dendritic cells (DCs)
- DCs can be targeted precisely and effectively in vivo using intravenously administered RNA-lipoplexes (RNA-LPX)
- Using well-known lipid carriers by optimally adjusting net charge, without the need for functionalization of particles with molecular ligands
- Future perspectives

Heinrich Haas, Vice President Drug Delivery, BioNTech RNA Pharmaceuticals

9.50 Advances in targeted delivery of anti-sense oligonucleotide therapeutics to pancreatic β -cells for regenerative approaches in Type 2 Diabetes

- Advantages of targeted delivery approaches based on GLP1-conjugation
- GLP1 peptide structure activity relationship with respect to gene knock-down in pancreatic β -cells
- Biodistribution of GLP1-conjugated and unconjugated anti-sense oligonucleotides (ASOs) and therapeutic window
- Possibility to utilise GLP1-ASO conjugates for regenerative approaches in Type 2 Diabetes

Shalini Andersson, Senior Director and Head of New Modalities, AstraZeneca

10.30 Morning Coffee

11.00 Delivering therapeutic oligonucleotides across biobariers: Opportunities and challenges in drug development

- Oligonucleotides have a huge pharmacological potential but their widespread therapeutic application is limited due to pharmacokinetic and drug disposition limitations at both the tissue and cellular level
- The presentation will address these delivery limitations and summarize the work of a European consortium (Innovative Medicines Initiative COMPACT Consortium) of pharma companies and academic partners to improve nanocarrier-based delivery technologies that can overcome these limitations

Ekkehard Leberer, Senior Director, Alliance Management, Sanofi

11.40 Advances in mRNA delivery technologies for vaccines

- Current state of delivery technology research for oligonucleotides
- mRNA vaccine opportunities in Infectious Diseases and Oncology
- Focus on delivery: design factors important specifically to mRNA vaccines

Marian Gindy, Executive Director, Pharmaceutical Sciences, Merck Research Labs, Merck & Co, Inc.

12.20 Delivering mRNA using polymeric and liposomal carriers

- Presentation and differentiation of polymer and liposome technologies
- Parameters for optimization towards in vivo applicability
- Data from preclinical models

Steffen Panzner, Managing Director, Lipocalyx GmbH

12.40 Networking Lunch

1.40 KEYNOTE: Exploring small activating RNA research at MiNA Therapeutics



- Huntington's disease is a fatal inherited neurodegenerative disease which currently has no cure
- Direct delivery to the cerebral spinal fluid are required since antisense nucleotides do not cross the blood brain barrier
- Pharmacodynamics and pharmacokinetics of huntingtin mRNA-targeting ASOs in larger brains when using intrathecal bolus injections

David Blakey, Chief Scientific Officer, MiNA Therapeutics

2.20 mRNA therapeutics for localized applications

- Stabilized non-immunogenic mRNA (SNIM®RNA)
- Formulations for mRNA delivery
- Pulmonary delivery
- Transcript-activated implants for bone regeneration

Carsten Rudolf, Chief Executive Officer, Ethris GmbH

3.40 Afternoon Tea

3.30 Case study: RNAi therapy for hATTR amyloidosis

- hATTR amyloidosis is an orphan condition caused by the deposition of mutant TTR protein into tissues, particularly the nervous system and cardiac system
- It causes severe morbidity and mortality
- Reducing the production of mutant TTR protein through RNA based therapeutics presents a novel way to treat this serious condition and this talk will focus on the role of an RNAi based therapy

Ali Murad, Director, Medical Affairs, Alnylam

4.10 SPOTLIGHT: Planning for oligonucleotide supply: personalized medicine to large scale markets



- Securing oligonucleotide drug substance supply
- Early vs late phase
- Small scale (ultra-orphan) vs large scale markets

Hans Kistemaker, Associate Director, ProQR

4.50 Chair's Closing Remarks and Close of Day Two

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