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SMi Group Proudly Present the 13th Annual Conference...

RNA Therapeutics

**Bolstering the latest advances and
opportunities in RNA-based medicine**

Venue to be confirmed, London, UK

FOCUS DAY: 8TH
CONFERENCE:
9TH - 10TH

FEB
2022



**Focus Day:
Vaccines in the RNA Industry**

8TH FEBRUARY 2022

FOCUS DAY CHAIR:



Steve Pascolo,
Founder and CEO,
Miescher Pharma

FEATURED SPEAKERS:

- **Derek O'Hagan**, Senior Advisor, Vaccines R&D, **GSK**
- **Arun Kumar**, Preclinical Vaccine Development Lead, **Coalition for Epidemic Preparedness Innovations (CEPI)**
- **Priya Karmali**, VP, Technology Innovation and Development, **Arcturus Therapeutics**
- **Wigard Kloosterman**, Chief Scientific Officer, **Frame Therapeutics**
- **Dr. Sun Woo Hong**, CEO of **mCureX Therapeutics**

CHAIR FOR 2022:



Heinrich Haas,
VP RNA Formulation and Drug Delivery,
BioNTech

FEATURED 2022 REGULATORY KEYNOTE:

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

FEATURED 2022 SPEAKERS INCLUDE:

- **Jimmy Weterings**, Principal Scientist Oligonucleotide Chemistry, **AstraZeneca**
- **Thomas Thum**, Founder and Chief Scientific Officer, **Cardior Pharmaceuticals**
- **Samir Ounzain**, CEO, **Haya Therapeutics**
- **David Lowe**, Vice President, Research, **Evox Therapeutics**
- **Alfica Sehgal**, Vice President, Head of Discovery, **CAMP4 Therapeutics**
- **Punit Seth**, Vice President, Medicinal Chemistry, **Ionis Pharmaceuticals**
- **Nagy Habib**, Head of HBP Surgery, Co-Founder, **Imperial College London and MiNA Therapeutics**
- **Dong Ki Lee**, CEO, **OliX Therapeutics**
- **Martin Akerman**, Chief Technology Officer & Co-Founder, **Envisagenics**

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LETTER FROM THE CHAIR:

Dear Colleagues,

It is with great pleasure and delight that I welcome you to SMI's highly anticipated RNA Therapeutics conference taking place on the 9th-10th of February 2022 in London.

The 13th annual conference will bring together expertise from individuals in multiple disciplines in the field addressing the key drivers of this rapidly growing industry. We have witnessed significant growth and innovation in the RNA landscape and this 2-day agenda will offer a series of presentations through which you will gain insights from leading pharmaceutical, biotech and regulatory representatives on the ever-growing field. Furthermore, with the heightened spotlight on mRNA vaccines in the fight against COVID-19, this year's pre-conference focus day will address vaccines and anti-virals in the RNA industry.

Delegates stand to gain many valuable insights from this event such as:

- Learnings and takeaways exploring the industry's pivotal role in the global pandemic
- Recent developments in novel and targeted RNA delivery systems
- Updates from leading pharmaceutical, biotech and regulatory representatives revolutionising the RNA therapeutics landscape
- Outlooks in advancing targets and target discovery for RNA therapeutics

As chair of this event and focus day, I look forward to welcoming you to this must-attend conference in London this coming February.

Yours sincerely,



Heinrich Haas,
VP RNA Formulation and Drug Delivery,
BioNTech

08.30 Registration & Coffee

09.00 Chairs' Opening Remarks

Heinrich Haas, VP RNA Formulation and Drug Delivery, **BioNTech**

ADVANCES IN RNA TECHNOLOGIES AND AN INDUSTRY OUTLOOK

OPENING ADDRESS

09.10 Reviewing the current environment of R&D and new technologies

- How have we seen the industry evolve over the past year?
- Significant developments in RNA technologies
- How can the recent acceptance of mRNA vaccines aid further advances in the industry?
- What are the major hurdles facing the industry and a look to new opportunities in the near future

Heinrich Haas, VP RNA Formulation and Drug Delivery, **BioNTech**

REGULATORY KEYNOTE

09.50 Policies relating to medicines and medical devices

- An insight into the MHRA's approach to mRNA vaccines over the past year
- MHRA updates and guidance on RNA therapeutics
- Regulatory advice on novel clinical trials

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

10.30 Morning Coffee

NOVEL AND TARGETED DELIVERY SYSTEMS

11.00 Exploring the chemistry of oligonucleotide therapeutics

- Examining further advances in chemistry that are earlier in the pipeline of oligonucleotide drug candidates
- Considering the possible effect of delivery systems that may provide extra footholds to improve the potency and specificity of oligonucleotide drugs
- Focusing on strategies to imbue antisense oligonucleotides with more drug-like properties and their applicability to other nucleic acid therapeutics

Jimmy Weterings, Principal Scientist Oligonucleotide Chemistry, **AstraZeneca**

11.40 Exosomes as a multi-modular vehicle for RNA Therapeutics

- Exosomes are naturally produced by all cells and play multiple roles in intercellular communication
- Exosomes are an exciting, emerging potential therapy for serious diseases
- At Evox we are engineering exosomes to deliver a wide variety of different RNA payloads in vivo

David Lowe, Vice President, Research, **Evov Therapeutics**

12.20 Networking Lunch

13.20 Preclinical to clinical transition of INT-1B3 (LNP-formulated miR-193a-3p mimic): initial lessons learned

- Moving from preclinical evaluation to first-in-human study
- Initial PK/PD correlation from preclinical to clinical findings
- Outlook: potential impact on clinical development acceleration

Michel Janicot, Chief Development Officer, **InterRNA Technologies**

SPOTLIGHT SESSION

14.00 Traversing protected tissue barriers for oligonucleotide delivery

- Current challenges in delivery of oligonucleotide therapeutics
- Insights into the use of Transferrin receptor ligands and other approaches to optimise delivery
- Case Study: Enhancing potency of oligonucleotide therapeutics in muscle tissues
- A future outlook for enhancing oligonucleotide delivery systems

Punit Seth, Vice President, Medicinal Chemistry, **Ionis Pharmaceuticals**



INDUSTRY DEVELOPMENTS IN RNA INTERFERENCE

14.40 A new era of programmable therapeutics to upregulate genes

- RNAs role in genome organization and gene expression and insight into the current use of regulatory RNAs in industry
- Targeting regRNA to increase gene expression and the use of RNA actuators to increase regRNA activity and restore levels of mRNA expression
- Understanding the technology behind programmable therapeutics for RNA targets in conjunction with RNA actuator technology
- Looking forward to pipelines in neurological and hepatocyte therapy

Alfira Sehgal, Vice President, Head of Discovery, **CAMP4 Therapeutics**

15.20 Afternoon Tea

15.50 Small activating RNA from concept to Phase 2 clinical trial

- Small activating RNAs (saRNA) are double stranded 21 nucleotide RNA that either target promoters or enhance genes leading to mRNA upregulation
- saRNAs can be delivered with liposomes into the systemic circulation or subcutaneously by conjugation with aptamers or GalNAC
- MTL-CEBPA is an investigative drug that resulted from the conjugation of saRNA CEBPA with NOV 340 liposomes that targets tumour associated macrophages in order to alter favourably the tumour microenvironment
- MTL-CEBPA has been administered safely in over 100 patients with advanced cancer and improved clinical outcome in a sub-set of patients when co-administered with TKI or checkpoint inhibitor

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

16.30 Therapeutic development using chemically modified asymmetric siRNAs

- RNA interference (RNAi)-based gene silencing has become the 3rd therapeutic development platform with multiple FDA-approved RNAi drugs on market
- OliX's asymmetric siRNA (asiRNA) triggers efficient target gene silencing with reduced non-specific effects
- OliX Pharmaceuticals has developed lipid-conjugated cp-asiRNA for local administration therapeutics, and GalNAC-asiRNA for liver targeting therapeutics
- I will describe OliX Pharmaceuticals' pre-clinical and clinical developments against various disease indications including (1) local administration therapeutics such as skin scar, androgenic alopecia, and age-related macular degeneration, (2) liver therapeutics such as hepatitis B virus (HBV), non-alcoholic steatohepatitis (NASH), and diabetes

Dong Ki Lee, CEO, **OliX Pharmaceuticals**

17.10 Chairs' Closing Remarks and Close of Day One

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08.00 Registration & Coffee

09.00 Chairs' Opening Remarks

Heinrich Haas, VP RNA Formulation and Drug Delivery, **BioNTech**

EXPLORING THERAPEUTIC TARGETS

09.10 OPENING ADDRESS

Programmable mRNA-targeting gene therapy to correct genetic diseases

- An introduction to CORRECTX: Locanabio's programmable RNA-binding protein platform
- Application of splicing modulation modality in ocular disease
- Emerging approaches for durable splice modulating therapeutics

Rea Lardelli, Senior Scientist, RNA Biology, **Locanabio**



09.50 Validation of therapeutic mode of action for heart failure program

- The landscape of microRNA for targeted therapy and miR-132's key role in cardiovascular diseases
- Investigating the molecular mechanisms of microRNAs in modulating cardiomyocyte contractility
- Understanding the study findings and the approach of blocking miR-132 to halt the process of cardiac disease
- What's next? Moving on to phase 2 studies

Thomas Thum, Founder and Chief Scientific Officer, **Cardior Pharmaceuticals**

10.30 Morning Coffee

11.00 Reimagining heart treatments via precision therapeutics

- Diving into HAYA's innovative tissue- and cell-selective genomic medicines
- How is the discovery engine using long coding RNAs to treat serious health conditions
- Identifying novel targets and drug candidates with potential for greater efficacy and safety
- Targeting Wisper and preclinical results showing the potential to reverse fibrotic processes and underlying heart failure

Samir Ounzain, CEO, **Haya Therapeutics**

11.40 Engineering the Future of mRNA Therapies

- Challenges and limitations of current mRNA technology
- Applications for a developing technology and the tools to do it
- The importance of process and supply

Volker Germaschewski, Senior Vice President of Research & Development, **eTheRNA Immunotherapies NV**



12.20 Networking Lunch

SPOTLIGHT SESSION

13.20 Systematic identification of novel microRNA targets for epilepsy treatment

- Temporal Lobe Epilepsy (TLE) and the therapeutic challenge – why do we need to address that?
- miRNAs as targets for epilepsy treatment - what do we know so far & what are we missing?
- EpimiRNA project: Systematic identification of novel miRNA targets for seizure suppression in TLE

Cristina Ruedell Reschke, Lecturer, School of Pharmacy and Biomolecular Sciences, **RCSI**



SPOTLIGHT SESSION

14.00 A novel microRNA target modulates voltage-gated sodium channels and may be a new therapeutic approach in genetic epilepsies

- We have identified a novel microRNA dysregulation in drug-resistant epilepsies
- This microRNA represses multiple voltage-gated sodium channels in the brain
- Manipulation of this microRNA can alter brain excitability and may be beneficial in genetic epilepsies

Gareth Morris, ERUK Emerging Leader Research Fellow, **University College London**



TARGET DISCOVERY FOR RNA THERAPEUTICS

14.40 RNA Therapeutics in Oncology: Advances and Challenges

- With the support of Envisagenics' proprietary machine learning modules, high performance computing, and splicing analytics, Envisagenics identified a novel target and designed and validated RNA therapeutic compound in 8 months
- Focusing on antisense oligonucleotides as a viable therapeutic modality in oncology and other applicable therapeutic areas
- Overcoming limitations to computational outputs and executing necessary validation experiments for in-silico identified targets

Gayatri Arun, Vice President of Biology, **Envisagenics**

15.20 Afternoon Tea

PANEL DISCUSSION

15.50 The Evolution and Expansion of RNA-based Therapeutics

- Defining applicable therapeutic areas for RNA-based therapeutics and the current promising and accepted disease areas
- Investor outlook on RNA therapeutics and their appetite for RNA-based companies
- Spectrum of RNA therapeutics (miRNA, ASO, mRNA, siRNA, etc.)
- Roles of small molecules in RNA therapeutics
- Modality comparison (small molecule vs. antisense; delivery vs. generalizable drugs) and pros and cons of RNA-based therapeutics

Panel Moderator:

Martin Akerman, Chief Technology Officer & Co-Founder, **Envisagenics**

Panelists:

Michel Janicot, Chief Development Officer, **InterRNA Technologies**

Thomas Thum, Founder and Chief Scientific Officer, **Cardior Pharmaceuticals**

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



16.30 Targeting the Noncoding Genome for the Development of RNA Therapies

- New concepts for the identification of tissue, cell-type, and disease specific regulatory ncRNA targets
- RNA-targeting modalities and approaches to tame the genome's dark side
- Translational potential of next-generation lncRNA targets

Samir Ounzain, CEO, **HAYA Therapeutics**

PANEL DISCUSSION

17.10 The Successes and Failures of RNA-based Therapeutics and Future Outlook

- Considerations of important characteristics and biology targeting RNA molecules directly.
- Pipeline and process overview of target selection criteria and ensuring therapeutic viability and specificity.
- Transforming antisense into a drug and taking sequence into consideration to understand potential off-target effects.
- Drug delivery considerations, chemistry considerations, and IP strategy
- Future outlook of RNA-based therapeutics (impact of COVID in the RNA field) and innovative companies in the last 10 years in this respective field

Panel Moderator: Martin Akerman, Chief Technology Officer & Co-Founder, **Envisagenics**

Panelists:

Sudhir Agrawal, Founder and President, **ARNAY Sciences**

Samir Ounzain, CEO, **HAYA Therapeutics**

Eric Lim, Associate Director Bioinformatics, **Stoke Therapeutics**



17.50 Chairs' Closing Remarks and Close of Day Two

MARKETING OPPORTUNITIES

Want to know how you can get involved? Interested in promoting your services to this market?

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Vaccines in the RNA Industry

Exploring the latest advances in vaccine development



Chaired by
Steve Pascolo, Founder and CEO,
Miescher Pharma

Focus day overview:

Part of the SMI's leading RNA Therapeutics Portfolio, this year will host a pre-conference focus day to explore and assess the latest advances of vaccines in the RNA industry. Leading industry figures will give an insight into the pivotal role of the RNA industry over the past year in the global pandemic, while further case studies will explore the vast therapeutic potential of mRNA-based vaccines. The focus day will open discussion for improved strategies to enhance vaccine development in the industry and industry outlooks will assess the potential of vaccine technology for future infectious diseases.

Topics Covered Will Include:

- **Hear** from leading industry experts on how the industry has adapted to develop lifesaving COVID-19 vaccine candidates
- **Discover** the power of mRNA-based vaccines for cancer therapy
- **Understand** the evolving technologies being developed to build the next generation of vaccines
- **Engage** in industry outlooks assessing the potential of vaccines in the RNA industry for future infectious diseases

08.30 Registration & Coffee

09.00 Chairs' Opening Remarks

Steve Pascolo, Founder and CEO, **Miescher Pharma**

OPENING ADDRESS

09.10 Synthetic mRNA vaccines

- History of synthetic mRNA vaccines
- Mode of action of natural and synthetic mRNA vaccines
- Production and formulation of synthetic mRNA vaccines
- Vaccines and therapies based on synthetic mRNA: now and in the future

Steve Pascolo, Founder and CEO, **Miescher Pharma**



LEARNINGS FROM THE VACCINES AGAINST COVID-19

SPOTLIGHT SESSION

09.50 Accelerated development of messenger RNA based COVID-19 candidate vaccines

- The development of mRNA-based vaccines is much faster compared to traditional approaches of vaccines development
- There are more than 18 vaccines under clinical evaluation and at least 25 under preclinical development
- mRNA platform technology promises to revolutionize future vaccines
- CEPI's current portfolio of mRNA-based vaccines and future directions

Arun Kumar, Preclinical Vaccine Development Lead, **Coalition for Epidemic Preparedness Innovations (CEPI)**



10.30 Morning Coffee

11.00 Innovative mRNA technology for COVID-19 vaccine development

- Introducing mCureX mRNA technology
- Discovery of an mRNA delivery system in a collaborative way
- Preclinical development of COVID-19 vaccine candidates

Dr. Sun Woo Hong, CEO of **mCureX Therapeutics**

mRNA-BASED CANCER VACCINES

11.40 Personalized mRNA vaccine for gastrointestinal cancer patients

- Insights from pre-clinical development of personalized mRNA vaccines for cancer patients
- mRNA vaccine-induced neoantigen-specific T cell immunity in patients with gastrointestinal cancer
- Assessing clinical data from early phase clinical trials
- The future of mRNA technologies and vaccines in oncology

Gal Cafri, Immunotherapy and Genetic Engineering Group Leader, **Sheba Medical Center**

12.20 Networking Lunch

13.20 Identification of Frame neoantigens for use in mRNA cancer vaccines

- A collaboration with eTheRNA Immunotherapies to develop a new generation of mRNA cancer vaccines
- Highlighting Frame Therapeutics' workflow to identify novel classes of neoantigens
- Presentation of immunological readout of Frame mRNA vaccines

Wigard Kloosterman, Chief Scientific Officer, **Frame Therapeutics**

VACCINE TECHNOLOGIES AND DELIVERY SYSTEMS

SPOTLIGHT SESSION

14.00 Assessing delivery systems for future mRNA vaccines

- An executive overview of delivery systems for mRNA vaccines
- Conceptual aspects of LNPs and other delivery systems
- Current examples of LNP delivery systems for mRNA vaccines
- Exploring the future of vaccine delivery technologies in the RNA industry

Heinrich Haas, VP RNA Formulation and Drug Delivery, **BioNTech**



14.40 Afternoon Tea

SPOTLIGHT SESSION

15.10 Using optimal technologies to build the next generation of vaccines

- The rapid advance of RNA vaccines
- The next generation of RNA vaccines
- Which vaccines can we build next with RNA
- The future role of vaccine adjuvants

Derek O'Hagan, Senior Advisor, Vaccines R&D, **GSK**



mRNA-BASED VACCINES FOR FUTURE INFECTIOUS DISEASES

15.50 The potential of mRNA-based vaccines for infectious diseases

- An insight into Arcturus' pipeline of vaccines and mRNA technologies
- Challenges in large-scale production of mRNA vaccines and how have we seen the industry develop and adapt in the past year
- Considerations for novel development and manufacturing technologies for mRNA vaccines and lessons learned from the COVID-19 vaccines
- Future mRNA vaccine development - how can we expect the industry to move forward in this space

Priya Karmali, VP, Technology Innovation and Development, **Arcturus Therapeutics**

16.30 Chair's Closing Remarks and Close of Focus Day

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eTheRNA immunotherapies is a private clinical-stage company driving mRNA technology and therapeutic product development to deliver an innovative generation of RNA chemistries, RNA process technologies and a new and advanced generation of therapeutic and vaccine products. eTheRNA uses its established R&D foundations and in-house GMP manufacturing facilities and builds on its proprietary mRNA, mRNA adjuvant and mRNA formulation platforms to progress technology innovation and product development.

eTheRNA was established in January 2013 as a spin-off company from the 'Vrije Universiteit Brussel' (VUB), following the development of its TriMix mRNA technology. www.etherma.be

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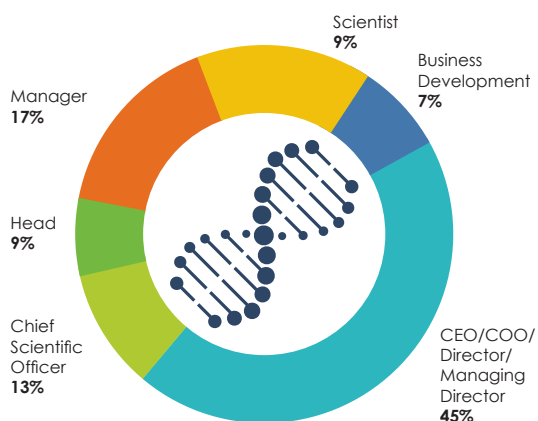
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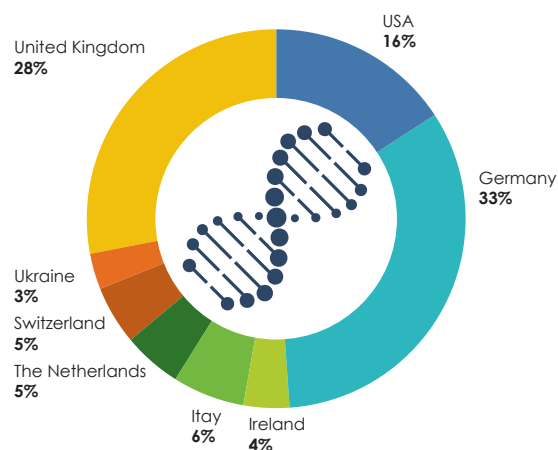
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RNA THERAPEUTICS

Conference: Wednesday 9th & Thursday 10th February 2022 **Focus Day:** Tuesday 8th February 2022
Venue to be confirmed, London, UK

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