A new paradigm for disease prevention

LSX are known for bringing life science investors together to assess and meet with the most promising up-and-coming biotech companies. When the first mRNA vaccines hit the market in 2020, one of our investors asked us to look at the resurgence of therapeutics targeting or made of RNA with the view to developing a high-level conference. And so, RNA Leaders was born.

Drawing on years of experience from running some of Europe's most respected life sciences events, we're excited to bring RNA Leaders to Basel in 2022. The conference will present the most interesting clinical data from biotech and pharma companies developing the next generation of RNA therapies and vaccines.

RNA medicines are at a critical moment in time. mRNA vaccines dominate the headlines in the wake of the COVID-19 pandemic. But huge advances are also being made in RNAi and antisense technologies. Our understanding of RNA chemistry continues to mature. Delivery tools are increasingly sophisticated and impactful. Advances in genomics and gene editing are driving new approaches. And exciting work targeting RNA with small molecules is emerging.

With that in mind, we can't wait to bring the community together, face-to-face, for the first annual RNA Leaders World Congress.

So, if you're passionate about all things RNA do join us, and more than 300 biotech, pharmaceutical, investment, regulatory and scientific professionals at the RNA Leaders World Congress. You'll hear the latest advances in RNA chemistry, get clinical updates from the companies developing new therapies and source the services partners who are helping to deliver drugs to patients.

Platinum partners

ChemGenes Corporation

CordenPharma

Precision NanoSystems

TriLink BioTechnologies, part of Maravai LifeSciences

Gold partners

abzu

LGC

AXO Labs

BIOORCHESTRA

cytiva

eTheRNA manufacturing

euroAPI Active Solutions for Health

Evonik Leading Beyond Chemistry

Janssen Pharmaceutical Companies of Johnson & Johnson

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ReCode Therapeutics

TATAA Biocenter

RNA Leaders World Congress 16-17 March 2022

Silver partners

Cend Therapeutics

REMIX Therapeutics

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Bronze partners

CODEX DNA

Eclipse BioInnovations

exopharm

eleven Therapeutics

Hongene Biotech Nucleosides & Nucleotides

Lipoid

NANOVATION therapeutics

PolyPeptide Group

PureHoney Technologies

Quantum Biosciences

rssl science with service

versameb

Supporting partners

Charles Consultants

FreeMind Non-Dilutive Funding Specialists

MEDiSTRAVA Consulting

Microbiology Society

Pharma network magazine

Scius Communications

SOPE society of physician entrepreneurs

zyme communications PR and Marketing for Life Science Companies

"This fresh approach to tackling genetic diseases has the potential to create a sea change in how we think about treating people with inherited conditions. With safe and effective treatments that really work, genetic diseases that are incurable at present need not be a life sentence in the future. The barriers that remain are not insurmountable. We still have work to do, but the future is bright for RNA therapies."

Lorna Harries, CSO, Senisca *

"The global antisense and RNAi therapeutics market is expected to reach $1.79bn by 2025 at a CAGR of 11%"

Research and Markets *

Meet the speakers

Nathaniel Wang
CEO, Replicate Bioscience

Nagy Habib
Founder and Head of R&D, MiNA Therapeutics

Marie Wikstrom Lindholm
SVP and Head of Molecular Design, Silence Therapeutics

Shalini Andersson
Head of Oligonucleotide Discovery, AstraZeneca

Tim Luker
Vice President, Venture Science, Corp BD, Eli Lilly, Eli Lilly

Alexandra Bause
Co-Founder, Investment Director & Head of VentureLabs, Apollo Health Ventures

Matthew Disney
Professor, Department of Chemistry, Scripps Research

Matthew Wood
Professor of Neuroscience and Deputy Head, Medical Sciences Division, University of Oxford

Thorsten Stafforst
Interfaculty Institute of Biochemistry, Eberhard Karls Universität Tübingen

Sylke Poehling
Senior Vice President and Global Head, Therapeutic Modalities, Roche

Sudha Chivukula
Head of RNA Technology, Research & External Innovation, Sanofi Pasteur

Malin Lemurell
Head of Medicinal Chemistry Early CVRM, Biopharmaceuticals R&D, AstraZeneca

David Slack
CEO, Cend Therapeutics

Branden Ryu
CEO, Biorchestra

Angele Maki
SVP & Head of Business Development, ReCode Therapeutics

Peter Smith
Co-Founder, President and CEO, Remix Therapeutics

RNA Leaders World Congress 16-17 March 2022

Meet the speakers

Dominik Witzigmann
Co-Founder and CEO, NanoVation Therapeutics

Douglas Fambrough
CEO, Dicerna Pharmaceuticals

Marc Abrams
Senior Vice President, Discovery Research, Dicerna Pharmaceuticals

Huw Nash
COO and CBO, Stoke Therapeutics

Suzanne Saffie-Siebert
Chairwoman and CEO at SiSaf

Bill Haney
CEO & Chairman, Skyhawk Therapeutics

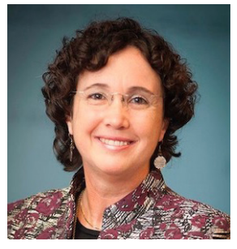
Kathleen McCarthy
Co-Founder & Chief Scientific Officer, Skyhawk Therapeutics

Lorna Harries
Co-Founder and CSO, Senisca

Aimee Jackson
CSO, Atalanta Therapeutics

Samir Ounzain
CEO and Scientific Co-Founder, HAYA Therapeutics

Lykke Pedersen
Head of RNA Therapeutics, Abzu



Laura Sepp-Lorenzino
CSO, Intellia Therapeutics

Dietrich Stephan
Founder and CEO, Neubase Therapeutics

Marcel Blommers
CSO, Saverna Therapeutics

Howard Stern
CSO, Korro Bio

Johannes Muhl
Senior Vice President, Finance, Exopharm

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Meet the speakers

Philipp Heller
Projektmanager Innovation, Evonik

Melissa Moore
CSO, Moderna

Denis Drygin
CSO, Regulus Therapeutics

Klaas Zuideveld
CEO, Versameb

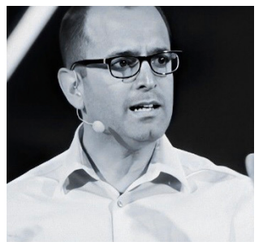
Hans-Peter Vornlocher
Managing Director, Research, LGC Axolabs

Brad Sorenson
Founder and Chief Executive Officer, Providence Therapeutics

Anastasiia Kamenska
Director, Corporate Strategy, Novartis

Martin Rabel
Field Application Scientist, Precision Nanosystems

Tamar Grossman
Global Head of RNA and Targeted Therapeutics Janssen



Yaniv Erlich
CEO, Eleven Therapeutics

Katarina Stenklo
Commercial Activation and End to End Solutions Leader, Cytiva

Bernard Sagaert
COO and Senior VP of Manufacturing, eTheRNA Immunotherapies

Siham Ceballos
Founder and Managing Partner, Bioartemis

Malgorzata Gonciarz
Associate Vice President, Genetic Medicine, Eli Lilly

Jason Coleman
Clinical Application Scientist Lead, Precision Nanosystems

Umberto Romeo
Head of R&D, Corden Pharma GmbH

RNA Leaders World Congress 16-17 March 2022

Meet the speakers

Alexandre Di Paolo
Business Development Senior Manager Trilink Biotechnologies

Kai Rossen
Chief Scientific Officer, EUROAPI

Scott Taylor
Associate Director, Alliance Management, Trilink BioTechnologies

Darach Neeson
COO, pHion Therapeutics

Prof. Helen McCarthy
CEO, pHion Therapeutics

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Agenda

Wednesday 16th March 2022
(All times in CET)

The bright future of RNA

Chair: **Tamar Grossman**, *Global Head of RNA and Targeted Therapeutics,* **Janssen**

08:30 Opening remarks

08:40 The technology that saved the world: how a once-dismissed idea led the fight against COVID-19

Prior to 2020 a vaccine had never been developed in less than four years. This session explores the remarkable story behind the development of mRNA vaccines for COVID-19, and what their success means for the future of RNA medicines.

- *How to take an unrealised idea and apply it to a new disease area*
- *Lessons learned from the rapid development of an experimental drug*
- *What does the success of mRNA vaccines mean for the future of RNA medicines?*

Melissa Moore, *CSO,* **Moderna**

09:10 Keynote panel – What will the next five years of RNA medicines look like?

After a decade of promise, RNA medicines are coming of age. This session presents the perspectives of RNA thought-leaders on the bright future of RNA, including where they see the most exciting growth and what continues to keep them awake at night.

- What lessons have been learned from clinical progress to date?
- Which new technologies offer exciting contributions to the field, and where are the major technology gaps still to be filled?
- What are the major challenges that the field still needs to solve to truly realise the potential of RNA medicines?

Speakers:
Melissa Moore, *CSO,* **Moderna**
Douglas Fambrough, *CEO,* **Dicerna Pharmaceuticals**
Bill Haney, *CEO & Chairman,* **Skyhawk Therapeutics**
Alexandra Bause, *Co-Founder, Investment Director & Head of VentureLabs,* **Apollo Health Ventures**

Moderator:
Siham Ceballos, *Founder and Managing Partner,* **Bioartemis**

09:50 Keynote panel – Where does RNA sit for Big Pharma?

Big Pharma's relationship with RNA has been fractious, but a handful of thought-leaders are driving internal and external programs to build RNA capabilities. This session explores the Big Pharma commitment to RNA medicines including partnership opportunities and research priorities for the next five years.

- Which RNA technologies are of the most interest and why?
- Assorted drug development strategies including internal R&D, partnership programs and external innovation
- What is the vision for this field in five to ten years?

Speakers:
Tim Luker, *VP, Search & Evaluation – Emerging Technology & Innovation, Corporate Business Development,* **Eli Lilly**
Shalini Andersson, *Chief Scientist, New Therapeutic Modalities and Head of Oligonucleotide Discovery,* **AstraZeneca**
Sylke Poehling, *Senior Vice President and Global Head, Therapeutic Modalities,* **Roche**
Tamar Grossman, *Global Head of RNA and Targeted Therapeutics,* **Janssen**

Moderator:
Kai Rossen, *Chief Scientific Officer,* **EUROAPI**

RNA Leaders World Congress 16-17 March 2022

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10:30 Morning Break and Oligonetworking Roundtables

Are you new to the field? Or would you like to meet some new players?

Join the Oligonetworking Roundtables to meet with other RNA leaders over a coffee.

A relaxed structured networking session for those who want to build new business relationships in this growing field.

Innovation Rapid Fire Session

The field of RNA is ripe for tackling new targets, modalities, technologies and delivery methods. This session presents a snapshot of some of the most exciting new work in early development from the next generation of RNA Leaders.

11:30 RNA therapeutics for the diseases of ageing

Levels of splicing factors change during ageing, compromising our ability to carry out "fine tuning" of gene expression. Senisca is using oligonucleotide-based therapeutic approach to reset splicing factor levels and reverse senescence.

Lorna Harries, *Co-Founder and CSO,* **Senisca**

11:50 Targeting long non-coding RNAs for tissue-specific treatment of fibrotic diseases

Specific long non-coding RNAs (lncRNAs) are important regulators of the resting fibroblast to myofibroblast conversion. HAYA is utilising modified ASOs to target and inhibit proprietary lncRNAs to prevent and reverse fibrosis.

Samir Ounzain, *CEO and Scientific Co-Founder,* **HAYA Therapeutics**

12:00 Regeneration of urethral muscle in Stress Urinary Incontinence using engineered mRNA

Engineered IGF-I-encoding mRNA following injection in the urinary sphincter resulted in secretion of therapeutically relevant protein levels which activated muscle regeneration. Treatment of stress urinary incontinence in an animal model resulted in functional and morphological regeneration, supporting its therapeutic potential.

Klaas Zuideveld, *CEO,* **Versameb**

12:10 Prophylaxis 2.0 via RNAi

The first generation of SARS-CoV-2 prophylaxis, namely mRNA-based vaccines, has been a remarkable scientific triumph. Yet, respiratory viruses continue to pose formidable challenges to all vaccine modalities. Eleven Therapeutics is developing Prophylaxis 2.0 via intranasal administration of RNAi therapeutics. It circumvents viral escape mutants, uniquely protects from inter-individual transmission, and shields at-risk populations such as immunocompromised conditions.

Yaniv Erlich, CEO, **Eleven Therapeutics**

12:20 Next-generation lipid nanoparticle technologies tailored to a variety of tissues.

The path to translate nucleic acid therapeutics into life-changing gene therapies is long, challenging, and uncertain. NanoVation Therapeutics is offering an LNP toolbox to enable safe and efficient delivery of nucleic acids to a variety of (extra)hepatic tissues as well as scalable production.

Dominik Witzigmann, *CEO & Co-Founder,* **Nanovation Therapeutics**

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12:30 Lunch

Delivery and distribution

Chair: **Johannes Muhl**, *Senior Vice President, Finance,* **Exopharm**

13:45 Chair remarks

13:50 Advancing extra-hepatic delivery of RNAi

With an advanced pipeline of RNAi therapies, Dicerna are making strides in the delivery of tissues beyond the liver. This session covers new leanings and gains in this field.

- Update on extrahepatic delivery platform technology
- Introduction to opportunities for RNAi in cancer immunotherapy

Marc Abrams, *Senior Vice President, Discovery Research,* **Dicerna Pharmaceuticals**

14:10 RNA therapeutics enable expansion of disease target space

RNA therapeutics are a rapidly expanding class of drugs that enable new disease targets and a new class of personalised medicines. This session looks at advances in targeted delivery and other work driving the field.

- Advances in targeted delivery of oligonucleotides beyond the liver
- Novel endosomal escape enhancers to improve oligonucleotide efficacy

Shalini Andersson, *Chief Scientist, New Therapeutic Modalities & Head of Oligonucleotide Discovery,* **AstraZeneca**

14:30 Lilly's view of genetic medicine & delivery strategies for RNA modalities

Malgorzata Gonciarz, *Associate Vice President, Genetic Medicine,* **Eli Lilly**

14:50 Harnessing tumor-targeting iRGD peptides to effectively target and deliver RNA-based therapeutics into solid tumors

While RNA-based drugs have reached market to help patients with a range of diseases, progress with this class of medicines for treatment of solid tumor cancers has been disappointing. The tumor stroma serves as a primary impediment to effective solid tumor delivery for many classes of drugs and particularly for RNA-based medicines. Cend Therapeutics is advancing a unique approach to convert the stroma from an impediment to a conduit for effective drug delivery.

- *Validation of lead investigational drug, CEND-1, in clinical trials for the treatment of pancreatic cancer – a tumor type particularly noted for its desmoplastic stroma*
- *CEND-1 is an internalizing RGD (iRGD) dual-acting cyclic peptide that enhances receptor-mediated endocytosis and transcytosis to penetrate all layers of the tumor*
- *Application of the same tumor-targeted tissue penetrating technology to enable effective delivery of RNA-based drugs within tumor-penetrating nanocomplexes (TPNs)*
- *Preliminary results for delivery on the enhanced iRGD-guided TPN delivery of RNA-based therapies.*

David Slack, CEO, **Cend Therapeutics**

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15:10 Overcoming the Blood–Brain Barrier for RNA therapeutics

Biorchestra is developing disease-modifying therapies for neurodegenerative diseases using RNA therapeutics and a proprietary polyion-complex-micelle-based delivery system, BDDS(TM).

How to deliver RNA therapeutics to the brain and brain cells
Tackling issues of deep brain delivery via intravenous injection
Introduction of our lead program adopting BDDS(TM)

Branden Ryu, *CEO,* **Biorchestra**

15:30 Afternoon Break and Services Clinics

Do you need specialised delivery technology, to build relationships with GMP providers or to get some IP advice?

In the Services Clinics participants are offered the chance to pre-book at 10-minute meeting with exhibitors and build business relationships with some of the leading service providers in the field.

Delivery and durability

16:00 Putting patients at the heart of an saRNA drug delivery strategy

RNA delivery, gene up-regulation with acceptable clinical outcome are not easy to achieve. This session takes on drug delivery and durability from a "patient-first" perspective, through the lens of RNA activation.

- Using RNA activation to restore normal function to cells
- Increasing duration of the drug, and why it matters
- RNA gene activation in the brain
- How to predict clinical response with small activation RNAs

Nagy Habib, *Founder and Head of R&D,* **MiNA Therapeutics**

16:20 CARTs - a new vehicle for nucleic acid delivery with a unique controllable release mechanism

CARTs are biodegradable, amphiphilic oligomers suitable for the formulation and delivery of nucleic acids. The session introduces this novel platform and gives an overview about the underlying concepts and application data.

- *CARTs are based on a polycarbonate-b-polyalphaaminoester backbone and self-assemble with nucleic acids into nanoparticles*
- *CARTs lead to a complementary biodistribution of RNA expression when compared to LNPs*
- *CARTs exhibit high transfection efficiency in various cell lines and are also active in hard to transfect cells such as primary T cells*

Philipp Heller, *Projektmanager Innovation,* **Evonik**

16:40 Delivering the Future of Genetic Medicines, Unconstrained by Delivery

The biotech and pharma industry has made great progress with the development of new modalities for novel genetic medicines. However, to fully capitalize on many of the new therapeutic modalities, encapsulation of all the necessary components and access to the intended target tissues are required.

ReCode has developed a proprietary selective organ-targeting (SORT) lipid nanoparticle (LNP) platform which enables targeted extrahepatic delivery of a variety of cargoes including mRNA and gene editing components.

Angele Maki, SVP & Head of Business Development, **ReCode Therapeutics**

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17:00 **Innovating the future of RNA therapeutics: silicon stabilized hybrid lipid nanoparticles (sshLNP)**

There are still a number of challenges that hold back the translation of RNA therapies into clinical reality. Perhaps the greatest of these challenges is the inherent instability of RNA molecules and their susceptibility to enzymatic degradation from RNAses. Chemical modifications to the RNA molecule itself and biochemical delivery platforms have so far only partially succeeded to address these challenges. SiSaf has developed silicon stabilized hybrid nanoparticles that overcome the limitations of current lipid nanoparticle technologies.

Benefits include:

·*Improved RNA stability*
·*Storage and distribution at ambient temperatures*
·*Enhanced endosomal escape and control release of RNA*
·*Increased payload capacity and reduced toxicity risk*

Suzanne Saffie-Siebert, Chairwoman and Chief Executive Officer, **Sisaf**

17:20 **Advances in RNA-targeted delivery approaches to access previously undruggable targets**

With the advancement of RNA-targeted therapeutics tries are being made in assorted delivery approaches. This session explores the development and application of several innovative approaches.

- Addressing limitations of current delivery platforms
- A look at innovative new approaches including antibody-olio conjugates, peptide-mediated delivery and exome-based nanotechnology
- Update on recent research and applications

Matthew Wood, *Professor of Neuroscience and Deputy Head, Medical Sciences Division,* **University of Oxford**

17:40 **Improving responses of mRNA using self-replicating RNAs**

Self-replicating RNAs have shown enhanced protein expression at lower doses than conventional mRNA vaccines, suggesting potential importance drug candidates. This session explores advancements in and applications of the technology.

- New developments in self-replicating RNA technology
- Applications for vaccines and mRNA therapies
- Role of self-replication in improving delivery and durability

Nathaniel Wang, *CEO,* **Replicate Bioscience**

18:00 **Famous Pairs Party**

Enjoy signature Margarita and Negroni cocktails, and canapés with prospective business partners at the Base Pairs networking Party.

Most importantly, can you find your 'base pair' at the party? Look out for your base pair on the bottom of your fellow attendee's badges. Attendees will carry an A, U, C or G on their badges.

Co-hosted and Welcome Remarks from TriLink Biotechnologies and CordenPharma.

Speakers:

Matthieu Giraud, *Director, Global Lipids and Carbohydrates Platform,* **Corden Pharma GmbH**
Simon Koerpert, *Director Business Development EMEA,* **TriLink BioTechnologies**

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Thursday 17th March 2022
(All times in CET)

RNAi advancements

Chair: **Anastasiia Kamenska**, *Director, Corporate Strategy,* **Novartis**

08:50 **Opening remarks**

09:00 **Lessons learned from preclinical and clinical development of microRNA therapies**

Dysregulated microRNA expression is a key factor in many complex multi-factorial diseases. This session explores Regulus's current pipeline of therapies in development including lessons during clinical progression.

- Update on preclinical and clinical pipeline
- Investigating new disease areas including delivery methods
- Regulatory challenges and key lessons for development of RNA therapies

Denis Drygin, *CSO,* **Regulus Therapeutics**

09:20 **A new modality to improve distribution, durability and potency of RNAi**

The need to treat neurodegenerative diseases is urgent and currently unsolved. This session explores a breakthrough therapeutic approach using RNAi to halt neurodegenerative conditions at their source.

- Understanding the modality including impact on distribution, durability and potency
- Update on pre-clinical development data
- Tackling delivery and distribution to the deep brain structure

Aimee Jackson, *CSO,* **Atalanta Therapeutics**

09:40 **Harnessing new chemistries and molecular design to silence disease-associated genes**

By harnessing the body's natural RNA interference mechanism, it is possible to create precision medicines that silence disease-associated genes. This session looks at new chemistries and molecular design to tackle a wide range of diseases.

- Putting patients at the heart of new discoveries
- Strategy for applying machine learning tools for siRNA selection

Marie Wikstrom Lindholm, *SVP and Head of Molecular Design,* **Silence Therapeutics**

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10:00 **Discovering RNA therapeutics with Abzu's explainable AI**

Two of the most promising advances in healthcare technology – RNA therapeutics and explainable AI – are fundamentally transforming what's possible in the field of therapeutic development. Abzu's pioneering bioinformatics platform effortlessly integrates heterogeneous data, such as sequences and multi-omics data, and reveals intricate nonlinear relationships – even on wide datasets.

The core technology, the QLattice, is the engine that generates a veritably infinite number of mathematical models to explain fundamental interactions – and yet identifying and understanding interactions is easy with straightforward and simple models. By using the QLattice, scientists gain a deeper understanding of biological and chemical mechanisms to create new drugs and modalities and increase their success rate, reducing the time required for drug discovery.

This presentation will show how Abzu's pioneering bioinformatics platform is being used to predict active siRNAs and how the explainable models uncover the underlying mechanisms in siRNA.

Lykke Pedersen, Head of RNA Therapeutics, **Abzu**

10:20 **mRNA-LNP Manufacturing Technologies – A Pilot Study Using a Microfluidic Approach**

LNPs are liposome-like structures especially geared towards encapsulating a broad variety of genetic payloads, primarily nucleic acids (DNA-RNA based) including siRNA, microRNA, mRNA and saRNA.

In late 2020, CordenPharma initiated a strategic investment program at the sterile injectable facility CordenPharma Caponago (IT) to develop a new LNP formulation process capability, as well as to have in-house analytical techniques to control the finalized LNP-assembled product.

With the present pilot study we challenged and optimized a NextGen Microfluidic manufacturing approach to generate high-yield and high-purity mRNA-LNPs designing a test formulation by using our lipid mixture.

Umberto Romeo, *Head of R&D*, **Corden Pharma GmbH**

10:40 **Morning Break and Oligonetworking Roundtables**

Are you new to the field? Or would you like to meet some new players?

Join the Oligonetworking Roundtables to meet with other RNA leaders over a coffee.

A relaxed structured networking session for those who want to build new business relationships in this growing field.

11:20 **RNA therapeutics modalities and targeted delivery approaches for developing transformational medicines for patients**

RNA therapies are an exciting and rapidly expanding category of drugs that have proven to speed solutions to the clinic and target previously undruggable pathways, for a broad array of therapeutic applications. The RNA and Targeted Therapeutics team at Janssen Research & Development is focused on developing RNA-based therapeutics and vaccines, using a broad range of modalities, including siRNA, ASO, saRNA and mRNA. The team is seeking targeted delivery solutions for safe, efficacious, and specific delivery to various tissues and organs.

The presentation will discuss Janssen's RNA efforts with examples of siRNA conjugated drugs for neurological disorders.

Tamar Grosman, *Global Head of RNA and Targeted Therapeutics*, **Janssen**

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mRNA therapeutics

11:40 **Developing a Transferable, Integrated mRNA Vaccine Platform**

mRNA vaccines, both prophylactic and therapeutic, have transformed the field of health care. This has been key to addressing the public health event of our lifetime, the COVID-19 pandemic. The latter has also brought focus on the hitherto ignored area of vaccine inequity.

In the presentation we will outline not only a robust, integrated, and scalable mRNA platform we have developed, together with a COVID-19 vaccine application, but also a unique business model premised on a collaborative network of global partners. This will help address access of mRNA products to the Global South and LMIC's at a fair and affordable price.

Brad Sorenson, Founder and Chief Executive Officer, **Providence Therapeutics**

12:00 **Idea to Injection: flexible and modular platforms to optimise your manufacturing**

The COVID-19 pandemic has accelerated the rapid development of mRNA vaccines, but this is just the tip of the iceberg for mRNA. mRNA has already been proven for use in cancer vaccines, immuno-oncology, localized regenerative injections to heal damaged tissues, and for producing systemic enzymes, proteins, and antibodies for a variety of diseases. Its application will only continue to grow in the future.

It is undeniable that mRNA is an innovative technology with much promise. However, investing in innovation brings uncertainties. What do you know about the product to be manufactured? What will the manufacturing scale be? Should you outsource or insource parts of the process?

In this presentation, we'll discuss strategies to address some of these uncertainties, demonstrating how investing in flexible design will make it possible to maximize output. Considering manufacturability and scale-up from the beginning will help to deliver process efficiency and scalability along the spectrum of mRNA products, from vaccines to therapeutics and beyond.

Katarina Stenklo, *Commercial Activation and End to End Solutions Leader*, **Cytiva**

12:20 **Things People Don't Talk About When Developing an RNA-based Infectious Disease Vaccine**

The current Covid—19 vaccines and the time to market timelines have led people to believe that developing an RNA based infectious disease vaccine is 'easy'.

In this presentation, we will highlight some of the decisions that need to be taken to get there, from the RNA chemistry encoded in the plasmids, through the process and production technologies needed to manufacture the drug substances and drug products and the required formulation technology to be able to administer the vaccine.

We will show how eTheRNA manufacturing can help to speed up the development based on platform technology that is available.

Bernard Sagaert, *COO and Senior VP of Manufacturing*, **eTheRNA Immunotherapies**

12:40 **mRNA Vaccines: Looking beyond the Pandemic**

mRNA Technology has led us to a new era in vaccinology offering rapid development of vaccines targeting a wide range of infectious diseases. Demonstrated successfully for COVID-19, this modality promises higher probability of success for a larger number of assets including complex multi-antigen products.

Additional research is ongoing to optimize mRNA design, intracellular delivery, and to bridge the knowledge gaps on tolerance and appropriate levels of immune stimulation for application beyond SARS-CoV-2 prophylaxis. The outline for screening novel mRNA designs and delivery towards obtaining balanced immune responses will be discussed. Pre-clinical evaluation of vaccine candidates against multiple viral pathogens and application of the technology in multi-antigen vaccine development will be presented.

Sudha Chivukula, *Head of RNA Technology, Research & External Innovation*, **Sanofi Pasteur**

13:00 Lunch

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Antisense approaches

14:00 **RNA-Based Therapeutic Modalities – A CRO's Perspective**

Over the last decade, we have seen a breathtaking number of novel therapeutic modalities and strategies in the area of oligonucleotide/nucleic acid/RNA-based therapeutics. This includes new modalities (e.g. single guide RNAs), new versions of "old" platforms (e.g. siRNA- and ASO-conjugates) and "light speed" maturation of new technologies (e.g. mRNA-vaccines).

These developments resulted in numerous approved therapeutics and a growing number of compounds in preclinical and clinical development. At the same time, new players with no "institutional" experience in the nucleic acid area entered the therapeutic area. This development raises significant challenges for custom-research and custom-manufacturing organizations. Novel approaches to identify lead candidates, to manufacture the compounds at different scales and to analyze/bio-analyze those new modalities must be in place in time.

Through case studies, we will present how Axolabs has been addressing those challenges and providing solutions to support the development programs of our clients.

Hans-Peter Vornlocher, Managing Director, Research, **LGC Axolabs**

14:20 **Upregulation of protein expression via ASO therapy**

Preclinical studies have shown that TANGO ASOs can be used to reduce the synthesis of non-productive mRNA and increase the synthesis of productive mRNA. This session explores the utility of the approach in developing mutation-independent therapies for autosomal dominant haploinsufficiency diseases.

- Understanding the technology
- Presentation of pre-clinical and clinical data
- Future activities and challenges

Huw Nash, *COO and CBO*, **Stoke Therapeutics**

14:40 **Drugging the genome to address base causality with a novel synthetic genetic medicine**

The use of a synthetic ASO platform offers the opportunity to practice genetic medicine at scale. The session explores how to combine the specificity of nucleotide engagement with the intracellular penetration and broad organ distribution capabilities of small molecules to target the genome or transcriptome.

- Understanding the technology including presentation of early data
- Addressing unique changes to the scaffold, targeting and self-assembly
- Applications and next steps

Dietrich Stephan, *Founder and CEO*, **Neubase Therapeutics**

Gene therapy and editing approaches

15:00 **Delivering on the promise of genetic medicine by co-opting the body's natural RNA editing system**

KorroBio aims to address rare genetic diseases as well as common illnesses by effecting precise, disease-modifying RNA edits with a simple oligonucleotide drug product. This session explores how oligonucleotides can be designed to re-direct the enzyme ADAR to make corrective or disease-modulating A to G edits using selected targets in the liver and central nervous system as examples.

- Understand the technology that enables precise RNA editing
- Understand how RNA editing differs from other genetic medicine approaches
- Illustrate the utility of the technology via presentation of pre-clinical data on targets in the liver and central nervous system

Howard Stern, *CSO*, **Korro Bio**

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15:20 **CRISPR as therapy: a non-viral approach to gene editing**

Using an LNP-based delivery system it is possible to selectively knock out disease-causing genes, introduce targeted insertion of a functional gene, or both. This session explores the potential of the system to treat genetic disease.

- Understanding the technology and its application
- Major challenges to be addressed
- Early clinical update and data presentation

Laura Sepp-Lorenzino, *CSO,* **Intellia Therapeutics**

15:40 **Harnessing ADAR-mediated RNA base editing for therapy**

Besides the genome, genetic information can be manipulated at the transcript level, including an approach called site-directed RNA editing. Particularly attractive for clinical translation is the harnessing of endogenous ADARs for adenosine-to-inosine RNA base editing. As inosine is biochemically interpreted as guanosine, genetic information is recoded.

- Strategies that harness the ubiquitously expressed, endogenous ADARs either with chemically modified ASOs or genetically encoded guideRNAs
- Compared to approaches with engineered ADARs, the latter gains advantage from providing only a guide RNA component and from very low off-target editing
- Overall, RNA base editing represents a promising opportunity for drug discovery.

Thorsten Stafforst, *Interfaculty Institute of Biochemistry,* **Eberhard Karls Universität Tübingen**

16:00 **Afternoon Break and Services Clinics**

Do you need specialised delivery technology? Or to build relationships with GMP providers? Or to get some IP advice?

In the Services Clinics participants are offered the chance to pre-book at 10-minute meeting with exhibitors and build business relationships with some of the leading service providers in the field.

Targeting RNA with small molecules

16:20 **Small molecule modulation of mRNA processing to control gene expression and target disease drivers**

mRNA processing is a highly regulated mechanism that controls gene expression and is known to be dysregulated in a wide variety of disease states. This session explores mechanisms of mRNA processing regulation and advances in the drug discovery toolkit that is leading to novel insights for small molecule discovery.

- *Understanding the complexity of mRNA processing mechanisms*
- *Developments in screening method*
- *Small molecule control of transcription factor expression in vitro and in vivo*

Peter Smith, *Co-Founder, President and CEO*, **Remix Therapeutics**

16:40 **Drugging the undruggable: correcting the underlying genetics of disease at the RNA level**

RNA mis-expression has been found to cause a growing list of diseases, from orphan and neurological conditions to major cancers. This session explores an approach to modify expression of target genes previously considered undruggable.

- A look at small molecule therapeutics for alternative modulation of RNA
- Update on Skyhawk's preclinical pipeline
- Major challenges still to be addressed

Kathleen McCarthy, *Co-Founder & Chief Scientific Officer*, **Skyhawk Therapeutics**

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Agenda

17:00 **Using small molecules for modulation of miRs relevant to cardiovascular, renal and metabolism diseases**

Drugging RNA using small molecules opens for new treatment avenues for patients. This session presents examples of using small molecules for modulation of miRs relevant to cardiovascular, renal and metabolism diseases.

- Synergies and complementarity to the rapidly evolving RNA therapeutic approaches
- Selectivity and functional response demonstrated in human relevant cell systems
- Development in screening assays

Malin Lemurell, *Executive Director and Head of Medicinal Chemistry Early CVRM,* **Biopharmaceuticals R&D, AstraZeneca**

17:20 **Early drug discovery of novel RNA drug targets**

By applying machine learning in combination with fragment-based screening using NMR, it is possible to revolutionize the lead-finding process. It enables the rapid validation of new RNA targets for the presence of valid binding sites for small molecules. Furthermore, starting points for chemical optimization can be identified within a very short time. This session examines how to apply AI in combination with experimental screening to identify novel RNA drug targets and inhibitors of these targets.

- Addressing the complexities of dynamic RNA molecules when identifying novel targets
- Application of RNA target selection and hit-to-lead generation in the SARS CoV-2 genome
- Application of hit-to-lead optimization in the bacterial ribosome

Marcel Blommers, *CSO,* **Saverna Therapeutics**

17:40 Close of congress

Workshops

Pre-event Workshop

Tuesday 15th March 16:00-19:00	Quantitative PCR for advanced therapeutics Hosted by TATAA Biocenter

Workshops

Thursday 17th March 09:00- 10:20	A Genomic Medicine Toolkit for Non-Viral Genetic Medicines: Payloads, Delivery and Manufacturing Hosted by Precision Nanosystems
Thursday 17th March 14:00- 15:30	Overcoming Hurdles in mRNA Manufacturing: Let's Scale Together Hosted by TriLink Biotechnologies

Workshops

Quantitative PCR for advanced therapeutics

Tuesday 15th March
16:00-19:00
Pullman Basel Europe Hotel
Clarastrasse 43, 4058 Basel, Switzerland

Hosted by TATAA Biocenter

Workshop leaders:

Mikael Kubista, CEO, TATAA Biocenter
Lydia Michaut, CSO, TATAA Biocenter

Scope of the Workshop:

- Ahead of the RNA Leaders World Congress, this is a unique opportunity to meet fellow biotech, pharmaceutical and scientific professionals and to learn about the recent expansion of TATAA's multi-omics analysis capabilities.
- The workshop will highlight recent developments in assay formats and show how TATAA can support biotech and pharma in achieving their R&D, pre-clinical, and clinical milestones according to the highest quality and regulatory standards.

Agenda:

16:00 Reception and coffee
17:00 Multi-omics approaches to drug discovery and development. Mikael Kubista, CEO, TATAA Biocenter
17:30 From COVID testing to mRNA therapeutics: quality first. Lydia Michaut, CSO, TATAA Biocenter
18:00 Networking Apéro

Please confirm your participation until Monday, March 7th by e-mailing to lukas.paul@tataa.com.

RNA Leaders World Congress 16-17 March 2022

Workshops

A Genomic Medicine Toolkit for Non-Viral Genetic Medicines: Payloads, Delivery and Manufacturing

Thursday 17th March,
9:00-10:20
Hosted by Precision Nanosystems

Workshop leaders:

Jason Coleman, Clinical Application Scientist Lead, Precision Nanosystems
Martin Rabel, Field Application Scientist EMEA Central, Precision Nanosystems

Scope of the Workshop:

- The workshop will provide an overview of the steps needed to bring an RNA Lipid Nanoparticle (LNP)-based genomic medicine from idea towards large scale manufacturing/GMP manufacturing
- The attendees will recive an overview of the Genomic Medicine Toolkit and how to use it to create non-viral genomic medicines covering: LNP formulation development as well as process development needed for LNP manufacturing including downstream processing methods and suitable analytical techniques
- The workshop will provide exemplary data and learning points based on a real-world example of an saRNA-LNP COVID-19 vaccine developed by Precision NanoSystems in Canada

Agenda:

Exploring the RNA-LNP Discovery Stage (30 minutes)
1. Introduction Round and Presentation of the Scope of Workshop
2. Introduction into the Genomic Medicine Toolkit
3. RNA-LNP Formulation Development Considerations
4. 1st Q&A Session covering points 1-3

Exploring the RNA-LNP Scale-Up Stage (45 min)

5. Analytical Requirements to Assess RNA-LNPs
6. 2nd Q&A Session to cover point 5
7. RNA-LNP Process Development Requirements and Scale-Up
8. Downstream Processing of RNA-LNP (TFF, Freezing and Storage)
9. 3rd Q&A Session to cover points 7 and 8

Session Wrap-Up and time-buffer for more questions (5 min)

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Workshops

Overcoming Hurdles in mRNA Manufacturing: Let's Scale Together

Thursday 17th March 2022
14:00 - 15:30
Hosted by Trilink BioTechnologies

Workshop leaders:

Scott Taylor, Associate Director, Alliance Management, Trilink BioTechnologies
Alexandre Di Paolo, Business Development Senior Manager GMP Europe, Trilink BioTechnologies
Prof. Helen McCarthy, CEO, pHion Therapeutics
Darach Neeson, COO, pHion Therapeutics

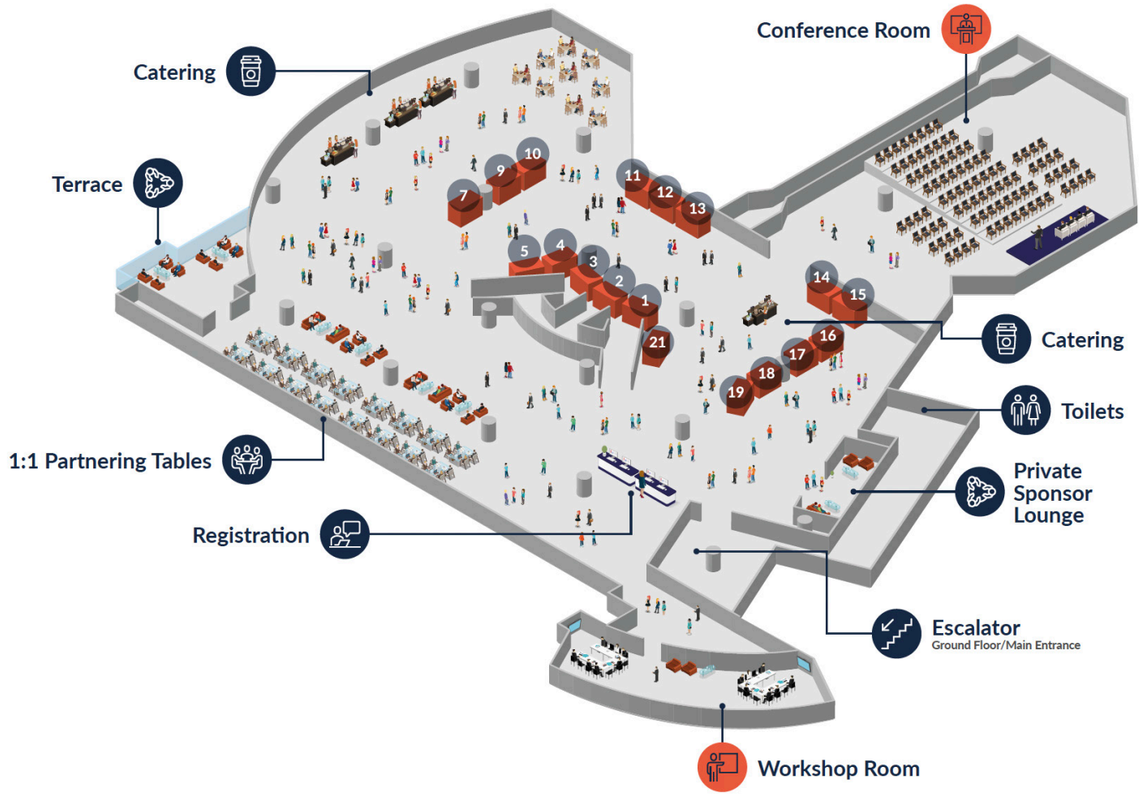
Scope of the Workshop:

The pandemic has accelerated acceptance, restored optimism, and helped lay the groundwork for developing safe and effective mRNA-based vaccines and therapeutics across different disease states.

Already, mRNA-related research is filling pipelines, and manufacturers are looking for efficient ways to make it on a large scale and for clinical-grade applications. However, the processes involved rely on highly technical expertise, limited materials, and accredited facilities. To that end, a vital component of mRNA-based vaccine and therapeutic development efforts is making informed decisions about manufacturing partnerships early.

Join us in this interactive workshop to learn about innovative solutions for mRNA production. Don't miss the opportunity to see what a path to partnership that scales with you at every stage of development looks like, including a case study and panel discussion with an industry partner who easily transitioned from RUO to GMP with TriLink BioTechnologies.

Floorplan and Exhibition Area



1. TriLink Biotechnologies
2. Precision Nanosystems
3. Corden Pharma
4. Sisaf
5. Quantoom Biosciences
7. Codex DNA
9. Hongene Biotech
10. RSSL Ltd.
11. EuroAPI
12. Cytiva
13. AxoLabs
14. Evonik
16. Purehoney Technologies
17. PolyPeptide Group
18. Biorchestra
19. eTheRNA Immunotherapies
21. Lipoid
22. Chemgenes

The Opportunity

500+ companies actively developing RNA therapeutics

4000+ live clinical trials for RNA therapeutics & vaccines

2 major mRNA vaccine approvals in 2020

3 RNAi therapeutic approvals in the past 4 years

1500+ investors actively looking to fund RNA technology

96 successful fund raises by mid 2021 (vs 101 total in 2020)

More than **$1.65bn** raised by RNA biotech mid 2021

Millions of lives saved by mRNA vaccines to date

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The Market

RNAi		Antisense technologies	Targeting with small molecule
Dicerna™	MiNA Therapeutics	IONIS	saverna therapeutics
Alnylam® PHARMACEUTICALS	SILENCE THERAPEUTICS	SAREPTA THERAPEUTICS	arrakis THERAPEUTICS
REGULUS	sirnaomics Advancing RNAi Therapeutics	STOKE THERAPEUTICS	SKYHAWK THERAPEUTICS
arrowhead pharmaceuticals	NeuroSense Therapeutics	TRIPLET THERAPEUTICS	REMIX THERAPEUTICS
Atalanta THERAPEUTICS		neubase	

RNA delivery		mRNA	Discovery tools
bicycle therapeutics	AVIDITY BIOSCIENCES	moderna	deep genomics
optimeos life sciences inc.	VesigenTx	BIONTECH	ENVISAGENICS
ARCTURUS therapeutics	PRECISION NANOSYSTEMS	CUREVAC the RNA people®	genuity science
Spartina Biotechnologies	vectalys by flash THERAPEUTICS	Translate BIO	
BIOORCHESTRA	Cend Therapeutics	Pantherna Therapeutics GmbH	

"Global mRNA vaccines and therapeutics market to reach $15.49bn by 2026, spurred by increasing investments in biotechnology"
imarc group *

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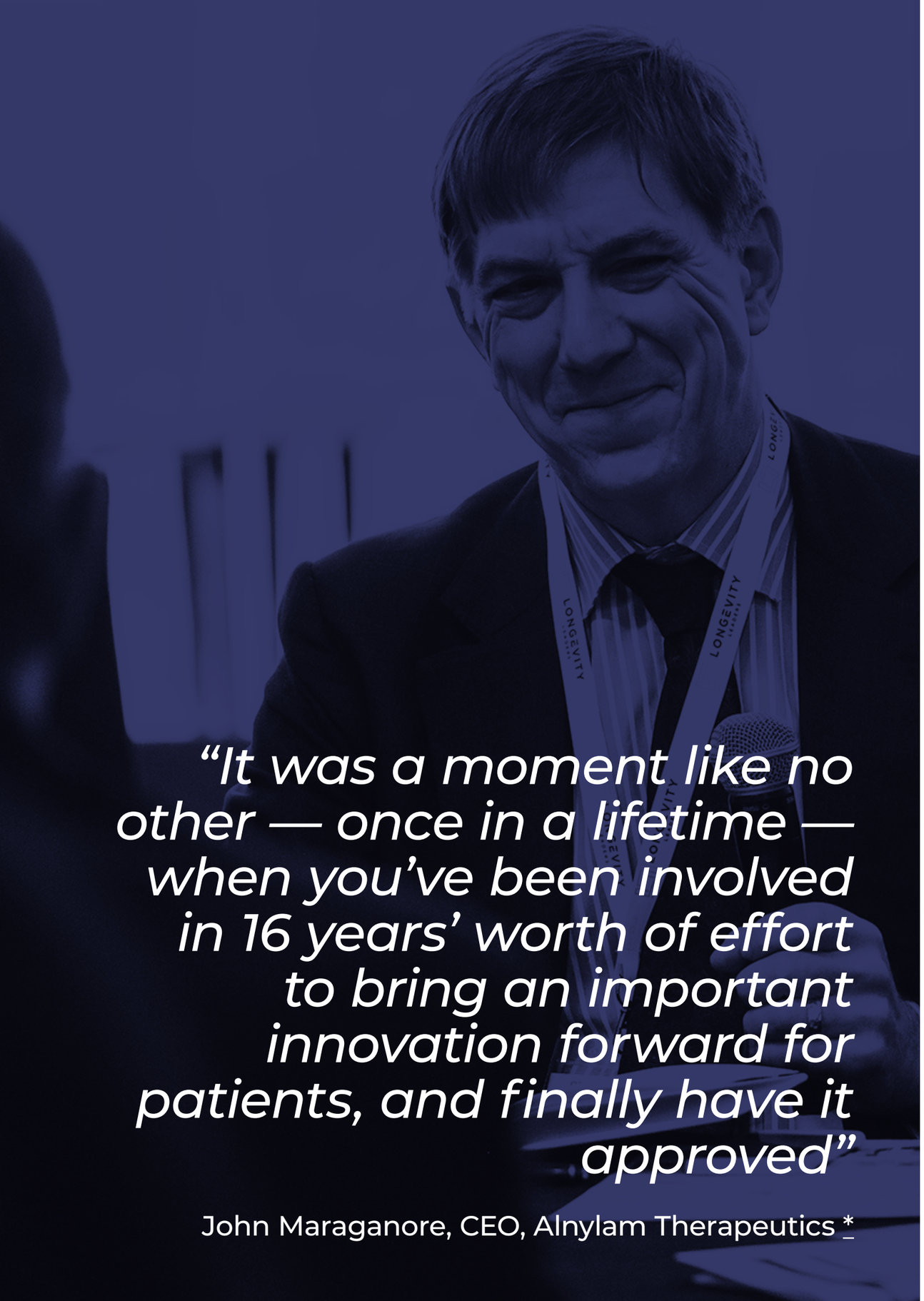

“It was a moment like no other — once in a lifetime — when you’ve been involved in 16 years’ worth of effort to bring an important innovation forward for patients, and finally have it approved”

John Maraganore, CEO, Alnylam Therapeutics *

Packages & pricing

Industry Tickets	Before 19-Nov	Before 17-Dec	Before 21-Jan	Before 18-Feb	Final Price
Standard Company Ticket · Two full conference days (16-17 March) · Access to networking app · Lunch & refreshments · Access to networking function	€1,890	€2,090	€2,390	€2,590	€2,790
Start-Up Biotech (<3yrs) Ticket · Two full conference days (16-17 March) · Access to networking app · Lunch & refreshments · Access to networking function	€1,140	€1,290	€1,440	€1,590	€1,740
Academic / Research Institute / NFP Ticket · Two full conference days (16-17 March) · Access to networking app · Lunch & refreshments · Access to networking function	€840	€890	€940	€990	€1,040

Discounts on tickets for groups

3+	save an extra 20%
5+	save an extra 30%

Contact marketing@lsxleaders.com for the **discount code!**

Book your tickets at www.lsxleaders.com/rna

Why Partner with RNA Leaders?

Proven track record

- 8 years of running life science events as a company
- More than 100 years of combined experience as a team

Commitment to good science

- Only the most interesting, responsible science presented
- Best-in-field industry and academic speakerss

Helping sponsors to present themselves well

- A continued, genuine working partnership
- Guidance on topics and materials

A valuable investor network

- Experience bringing 100s of investors to events
- Profiling helps to match investors with companies

Virtual and physical experience

- Known for best-in-class event delivery
- Successful integration of virtual elements with physical

First movers in an emerging field

- Bringing people face-to-face again
- Showcasing developments across all RNA medicines

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Why sponsor?

Thought Leadership

- Put your experts on stage with other RNA leaders
- Present your company as the partner-of-choice

Education

- Explain your unique service or technology live on stage
- Take time to present new data and strengthen your claims

Branding

- Ongoing promotion of your logo
- Promotion of your company on social channels

Business Development

- Showcase your technology or services
- Send your sales team to develop relationships with C-suite

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Our attendees are looking for:

Delivery platforms	Target discovery tools	RNA editing tools
Contract research	CDMO and CMC	RNA analysis
Equipment supply	Reagent providers	Legal services

If you offer products or services in any of these fields, our biotech and pharmaceutical attendees want to meet you.

To take part, contact joe@lsxleaders.com

Sponsorship packages

Benefits	Platinum	Diamond	Gold	Silver	Exhibitor
Thought Leadership					
Keynote speech	One of	One of			
Keynote panel	One of	One of			
Education					
Pre-event webinar	X	X			
Track speech	One of		X		
Track panel	One of			X	
Branding					
Event website	X	X	X	X	X
Event marketing	X	X	X	X	X
Social media	X	X	X	X	X
Onsite at event	X	X	X	X	X
Business Development					
Staff passes	6	4	3	2	
Client passes	6	4	3	2	
Exhibition booth	2x3m	2x3m	2x3m	2x3m	2x3m
Investment Level	~~£££££~~	~~££££~~	~~£££~~	~~££~~	~~£~~

Bespoke sponsorship packages including CEO Forum, Drinks Reception and premium branding opportunities are available. Contact joe@lsxleaders.com to find out more.

Contact the team

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Or visit us at
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