January 28-30, 2025 | Boston, MA
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WELCOME
EXPERT SPEAKERS
AGENDA
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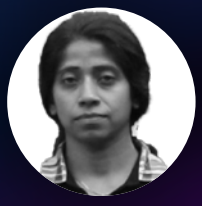
6th Annual

RNAi-Based Therapeutics Summit

Accelerating the Clinical Approval of the Next Wave of RNAi Medicines

Unlocking Novel Targets, Advancing Extra-Hepatic Delivery & Optimizing Chemical Modifications to Improve Selectivity, Durability & Bioavailability of the Next Generation of RNAi Medicines

Expert Speakers Include:



Shanthi Ganesh
Director, Global Nucleic Acid Therapies
Novo Nordisk

Muthiah (Mano) Manoharan
Senior Vice President, Innovation Chemistry & Alnylam Distinguished Scientist
Alnylam Pharmaceuticals

Zhihong Huang
Associate Director, Global Discovery Chemistry
Novartis

Adam Dinerman
Senior Vice President, Head of CMC
Aro Biotherapeutics

Alfica Sehgal
Chief Scientific Officer
Judo Bio

Hanhua Huang
Vice President, Biology
Avidity Biosciences

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Welcome to the 6th RNAi-Based Therapeutics Summit

6th Annual RNAi-Based Therapeutics Summit

Following Q4 2023 observing the approval of the 6th ever RNAi medicine, it is not surprising that this dedicated community continue to see waves of even more promising advances. 2024 saw **Rona Therapeutics secure $35m to advance their siRNA pipeline** and RNAi giant **Sirnaomics announce a partnership with gore range capita to advance its RNAi therapeutics into aesthetic medicine**.

The **6th RNAi-Based Therapeutics Summit 2025** returns to Boston as the only dedicated forum for all things RNAi, empowering leaders to progress their RNAi candidates into clinically successful therapies. This forum will unite the leading minds of the RNAi community to share **breakthroughs in delivery technologies such as AAV**, **AOCs and ligand-mediated conjugates**, **cutting-edge backbone chemistry** and **CMC innovation** for the development of the next generation of RNAi medicines.

With an expert program curated with insight from **Benitec Biopharma**, **Alnylam Pharmaceuticals**, **Novo Nordisk** and **Avidity Biosciences**, this biopharma dedicated meeting will showcase the latest data and advances in RNAi therapeutic development, focusing on:

- Improving selective delivery, targeted endosomal escape, tumor bioavailability and stability of RNAi payloads to create durable, tolerable therapies
- Expanding the therapeutic potential of RNAi medicines by reaching extra-hepatic targets including placenta, muscle and kidney to treat diseases with unmet medical need
- Streamlining platform manufacturing, devising analytical control strategies and maintaining product quality to develop efficient processes which support clinical success

Join 70+ RNAi experts from **discovery**, **research**, **translation** and **manufacturing** for 3 days of data-driven insights and strategies to **drive the next generation of RNAi drug development** and **bring tolerable**, **selective therapies** to patients in need.

70+ **Attendees**

27+ **Speakers**

21+ **Data-Driven Presentations**

3 **Interactive Workshops**

1 **Scientific Poster Session**

KEY BENEFITS OF ATTENDING

Maximize bioavailability and precision targeting to a wider range of tissues by seizing the value of novel delivery systems beyond GalNac, with insights from **SaneGeneBio**, **Avidity Biosciences** & **Sapreme Technologies**

Reach the full potential of RNAi therapy by reaching previously undruggable extra-hepatic targets including the CNS, placenta, muscle and kidney, with data from **Comanche Biopharma**, **Novo Nordisk** & **Judo Bio**

Advance stable, precise delivery with reduced off-target effects by employing cutting edge generative AI and deep-learning based platforms to revolutionize RNAi design and delivery, with insight from **Nosis Bio**, **Tallas Biotech** & **ProteinQure**

Enhance the stability, efficacy and tolerability of RNAi payloads through clever chemical modifications which suppress off-target activity and improve durability with research from **Novartis**, **Stony Brook University** & **Alnylam Pharmaceuticals**

Increase success of early-stage RNAi platforms and assets and attract investment and meaningful partnerships to progress your RNAi research into clinically successful ventures, with insight from **Sunrise Bioventures, Eli Lilly & Broadview Ventures** & **Broadview Ventures**

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What's New For 2025?

6th Annual RNAi-Based Therapeutics Summit

BRAND-NEW COMPANIES & ACADEMIC INSTITUTIONS

Showcasing emerging data on extra-hepatic targeting, AI-based drug design and innovative delivery strategies, 2025 brings you a brand-new, all-star line-up of expert Speakers from the RNAi space, uniting to drive the next wave of RNAi therapeutic development.

BENITEC BIOPHARMA silencing genes for life · NEXTGEN HEALTHCARE SOLUTIONS Bound Therapeutics, LLC · BROADVIEWVENTURES · ceria THERAPEUTICS · ProteinQure

COMANCHE BIOPHARMA · judobio · KORRO BIO · Lilly · NOSIS BIO

NOVARTIS · PepGen · SanegeneBio · Stony Brook University

NEW DISEASE FOCUS AREAS IN 2025

Advancing the scope of RNAi therapeutic potential, 2025 will bring new data on a range of diseases and targets beyond the liver, including preeclampsia, breast cancer and fibrodysplasia ossificans progressiva.

Showcasing novel RNAi therapeutic targets including:

Kidney · Immune cells · Tumor cells · Muscle · Placenta · CNS · Adipose tissue

BRAND NEW, UNPUBLISHED DATA IN 2025

The **6th RNAi Summit** will showcase new and unpublished preclinical and clinical data on novel delivery technologies, clever chemical modifications and innovative extra-hepatic targeting from some of the sharpest-minds in the field:

Julia Alterman
Assistant Professor
UMass Chan Medical School
Applying Hydrophobically Conjugated siRNA to the Treatment of Muscular Diseases

Adam Dinerman
Senior Vice President, Head of CMC
Aro Biotherapeutics
Centyrin-siRNA Conjugates: CMC Development Path to Clinic

Ruben Postel
Chief Scientific Officer
Sapreme Technologies
Improving Targeted Endosomal Escape for More Potent & Durable Therapies

Shanthi Ganesh
Director, Global Nucleic Acid Therapies
Novo Nordisk
Targeting Tumor-Associated Immune Cells with RNAi-Lipid Conjugates

Your Expert Speakers

Glenn Kazo
Chief Business Officer
ARIZ Precision Medicine

Muthiah (Mano) Manoharan
Senior Vice President, Innovation Chemistry & Alnylam Distinguished Scientist
Alnylam Pharmaceuticals

Adam Dinerman
Senior Vice President, Head of CMC
Aro Biotherapeutics

Zhanna Druzina
Senior Director
Aro Biotherapeutics

Hanhua Huang
Vice President, Biology
Avidity Biosciences

Hunghao Chu
Associate Director
Benitec Biopharma

Eriks Rozners
Professor
Binghamton University

Yuanyuan Jin
Co-founder & Chief Operating Officer
Bound Therapeutics

Benjamin Kreitman
Principal
Broadview Ventures

David Jackson
Chief Executive Officer
Ceria Therapeutics

Vignesh Narayan Hariharan
Director of Biology
Comanche Biopharma & Instructor
UMass Chan Medical School

Dallece Curley
Associate Director of External Innovation
Eli Lilly

Alfica Sehgal
Chief Scientific Officer
Judo Bio

Mohammad Shadid
Vice President, Preclinical Development
Korro Bio

David Sharp
Chief Executive Officer
MicroCures

Chad Miller
Head of Research
Nosis Bio

Zhihong Huang
Associate Director, Global Discovery Chemistry
Novartis

Shanthi Ganesh
Director, Global Nucleic Acid Therapies
Novo Nordisk

Ashling Holland
Director of Research and Preclinical Development
PepGen

Lucas Siow
Chief Executive Officer & Co-founder
ProteinQure

Weimin Wang
Founder & Chief Executive Officer
SanegeneBio

Ruben Postel
Chief Scientific Officer
Sapreme Technologies

Jingfang Ju
Professor
Stony Brook University

Andrew Fraley
Partner
Sunrise Bioventures

Ali Shah
Co-founder & Chief Executive Officer
Tallas Biotech

Zdravka Medarova
Chief Scientific Officer
TransCode Therapeutics

Julia Alterman
Assistant Professor
UMass Chan Medical School

Dr. Pablo Lores Lareo
CMC Director
Sylentis

Pre-Conference Workshop Day

Tuesday, January 28, 2025

6th Annual RNAi-Based Therapeutics Summit

Check In & Light Breakfast 8.00

Workshop A 9.00

Maximizing Bioavailability of RNAi Therapies with Oligonucleotide Conjugates to Reach Previously Undruggable Therapeutic Targets

Whilst the hunt for next-generation delivery platforms continues, and the scope for targeting extra-hepatic tissue increases, the success of RNAi therapeutics is limited if bioavailability is not addressed. For RNAi therapies to successfully induce gene silencing, the payload must be released effectively to the target tissue. As such, this workshop will address:

- Utilizing machine learning and algorithms to identify targets and design precise siRNA sequences
- How siRNA conjugates can improve targeted delivery and bioavailability in extra-hepatic tissue
- How to select optimized delivery systems which amplify bioavailability whilst maintaining target specificity

Workshop Leaders

Alfica Sehgal
Chief Scientific Officer
Judo Bio

Morning Break & Networking 11.00

Workshop B 11.30

Streamlining Translation & De-risking RNAi Therapeutic Development with Optimal Selection of Preclinical Models & Appropriate Assays to Ensure Tolerable, Potent Therapies

There are many promising RNAi therapeutic candidates at both the preclinical and clinical phase of development, but few drugs have yet been approved. To maximize clinical success, the right preclinical models should be used, mimicking the genotype of human patients. Assays to determine toxicity and efficacy must be precise, and dosing should be considered. Therefore, this Workshop will address:

- How to derisk novel chemical modifications in in vitro and in vivo preclinical models to safeguard against off-target effects and select animal models which mimic human genotype and pathology
- How to identify assays to determine toxicity, immuno-stimulation and therapeutic potency
- How to plan effective clinical trials considering human dose projections, PK and toxicity
- How to consider IND filing and regulatory approval throughout the translational pipeline

Workshop Leaders

Mohammad Shadid
Vice President, Preclinical Development
Korro Bio

Hanhua Huang
Vice President, Biology
Avidity Biosciences

> "This meeting allows me to gain insight from industry peer experiences and track new developments in extra-hepatic delivery of oligonucleotides"
>
> **Senior Vice President, Head of CMC, Aro Biotherapeutics**

Pre-Conference Workshop Day

Tuesday, January 28, 2025

Lunch Break & Networking 1.30

Workshop C 2.30

Seizing the Value of RNAi Therapeutics for Oncology to Develop Precise, Effective Treatments for Patients

RNAi therapeutics holds promise for treating complex genetic diseases such as cancer by targeting multiple oncogenes, but release of the therapeutic payload must be rapid and precise, and the heterogeneity of tumors must be considered. Therefore, this Workshop will address:

- How to identify genetic targets in heterogenous tumors to increase correlation in response at the preclinical and clinical stage
- How to design miRNA to target multiple oncogenes to overcome tumor resistance and limit toxicity
- How to integrate chemotherapeutic nucleoside analogs into miRNA to amplify tumor killing and induce a durable anti-cancer response
- How to stimulate release of siRNA from the endosome to invoke a rapid response against tumor cells

Workshop Leaders

Jingfang Ju
Professor
Stony Brook University

Glenn Kazo
Chief Business Officer
ARIZ Precision Medicine

Zdravka Medarova
Chief Scientific Officer
TransCode Therapeutics

End of Pre-Conference Workshop Day 4:30

"Sharing cutting edge and novel approaches towards enhancing RNA editing therapeutics to bring drugs to the clinical and to people in need in a safe, robust and timely manner is essential"

Director of Research and Preclinical Development, PepGen

"Exchange of ideas and results with field experts will facilitate deliveries of much needed therapies for the patients"

Senior Director, Aro Biotherapeutics

Conference Day One
Wednesday, January 29, 2025

7.50 **Check In & Light Breakfast**

David Jackson
Chief Executive Officer
Ceria Therapeutics

8.50 **Chair's Opening Remarks**

Successfully Targeting Extra-Hepatic Tissues with Novel RNAi Therapeutic Payloads to Expand the RNAi Treatment Paradigm

Shanthi Ganesh
Director, Global Nucleic Acid Therapies
Novo Nordisk

NEW DATA

9.00 **Targeting Tumor-Associated Immune Cells with RNAi-Lipid Conjugates**

- Refractory malignant solid tumors create an immunosuppressive TME which renders them resistant to standard-of-care immune checkpoint inhibitors
- How RNAi agents can silence CD274 (PD-L1) in tumor-associated immune cells, which mediate immune suppression in the TME
- Silencing Cd274 in antigen presenting cells remodeled the TME, increased cytotoxic T-cell infiltration and mediated single agent activity in immunotherapy resistant pre-clinical tumors. Human active PDL1 RNAi is in currently in Phase 1 clinical trials for immunotherapy-refractory cancers (NCT06504368)

David Sharp
Chief Executive Officer
MicroCures

UNPUBLISHED DATA

9.30 **Novel siRNA Platform for Cellular-Driven Tissue Repair & Regeneration**

- Fidgetin-like 2 in cell migration
- Fidgetin-like 2 as a target for tissue repair
- Localized siRNA delivery for the treatment of traumatic injury

Alfica Sehgal
Chief Scientific Officer
Judo Bio

10.00 **Receptor Mediated Targeted Delivery to Proximal Tubules in Kidney**

- Complexity of the kidney as a tissue and the challenges of targeting kidney
- Our work with ligands for specific targeting in kidney cell types
- Insights into receptor biology and uptake of conjugated oligos

10.30 **Morning Break & Speed Networking**
This is your opportunity to get face to face with some of the biggest names in RNAi therapeutics to connect and establish meaningful industry relationships.

Reaching Previously Undruggable Tissues through Identification of Tissue-Specific Cell Receptors to Expand the Therapeutic Potential of RNAi

Julia Alterman
Assistant Professor
UMass Chan Medical School

UNPUBLISHED DATA

11.30 **Applying Hydrophobically Conjugated siRNAs to the Treatment of Muscular Diseases**

- A combinatorial treatment strategy for reducing heterotopic bone in a mouse model of Fibrodysplasia Ossificans Progressiva (FOP)
- Identification of a SNP-targeting siRNA against the mutant ACVR1 receptor implicated in FOP
- Systemic delivery of hydrophobic siRNAs to the muscle

Zhanna Druzina
Senior Director
Aro Biotherapeutics

NEW DATA

12.00 **A Novel Platform to Discover Targeting Proteins for siRNA Delivery into Immune Cells**

- Development of new protein libraries with expanded diversity to select ideal targeting proteins for extra-hepatic siRNA delivery
- Targeting proteins from new libraries using transferrin receptor specific binders that enabled siRNA delivery and gene knockdown in skeletal muscle
- In vitro and in vivo validation of novel immune cell specific -siRNA conjugates

Conference Day One
Wednesday, January 29, 2025

Vignesh Narayan Hariharan
Director of Biology
Comanche Biopharma
& Instructor
UMass Chan Medical School

12.30 **Delivery of siRNA to the Placenta for the Treatment of Preeclampsia**

- Identification of sFLT1, a potential therapeutic target for the treatment of preeclampsia
- Optimization of conjugates for placental uptake
- Developing chemical technology for the silencing of placental sFLT1

1.00 **Lunch & Networking**

Integrating Novel Chemical Modifications to Enhance the Stability, Potency & Delivery of RNAi Therapies for Improved Therapeutic Intervention

Muthiah (Mano) Manoharan
Senior Vice President, Innovation Chemistry & Alnylam Distinguished Scientist
Alnylam Pharmaceuticals

2.00 **Living in the World of RNA Therapeutics made possible by Novel Chemical Modifications**

- Talk Details To Be Confirmed

Eriks Rozners
Professor
Binghamton University

UNPUBLISHED DATA

2.30 **Amide-Modified RNA: Using Protein Backbone to Improve Specificity of siRNAs**

- Amides are excellent mimics of phosphate linkages in RNA and are well tolerated in the guide strand of siRNAs
- Amide modification at the 5'-end (position 1) of the passenger strand suppresses its undesired off-target activity
- Amide modification in the seed region of the guide strand suppresses undesired microRNA-like off-target activity

Jingfang Ju
Professor
Stony Brook University

NEW DATA

3.00 **Development of Novel Chemical Modification Strategies for miRNA and siRNA Based Cancer Therapeutics**

- Novel chemical modifications to enhance stability of miRNA and siRNA
- How specific chemical modifications can reduce and eliminate the requirement of delivery vehicle for delivering RNAi therapies
- How to improve therapeutic efficacy using innovative chemical modifications

3.30 **Roundtable Discussion: How Can We Design Better RNAi Payloads? Enhancing Stability & Maximizing Precision Targeting of RNAi Therapies through Chemical Modifications**

- How can alternative siRNA structures alter therapeutic delivery, efficacy and stability
- How to employ nucleoside modifications to ligands to influence therapeutic stability
- What innovative biochemical strategies are providing scope to improve RNAi therapies

Zhihong Huang
Associate Director, Global Discovery Chemistry
Novartis

Eriks Rozners
Professor
Binghamton University

4.00 **Afternoon Break & Poster Session**
This is your opportunity to share your ground-breaking data and innovative delivery strategies with your fellow RNAi experts.

Employing AI & Computational Modelling to Design Tolerable RNAi Therapies to Target Specific Tissues with Improved Efficacy & Durability

Ali Shah
Co-founder & Chief Executive Officer
Tallas Biotech

NEW DATA

4.30 **Using Generative AI to Design Novel Synthetic RNAi Molecules**

- How AI can support rational drug design
- Reviewing legacy Tallas AI models and approaches
- Projections for AI in drug discovery

Conference Day One
Wednesday, January 29, 2025

Chad Miller
Head of Research
Nosis Bio
UNPUBLISHED DATA

5.00 **Deep Learning-based Design of Cell-targeting Ligands Enables Cell-specific, Functional siRNA Delivery in Multiple Extra-hepatic Tissues**

- Receptor-mediated endocytosis drives cell tropism across RNA modalities
- Deep learning-based design of ligands targeting endocytosing receptors enables rapid in vivo testing of receptors for their capacity to mediate functional siRNA delivery
- This platform – Connexa – has led to the discovery of multiple receptors capable of mediating *in vivo* functional siRNA delivery in extrahepatic tissues

Lucas Siow
Co-founder & Chief Executive Officer
ProteinQure

5.30 **AI Design of Targeted Ligands for Extra-hepatic siRNA Delivery**

- How AI and other computational tools can help design peptides for tissue specific delivery of siRNA
- How peptide based delivery systems can allow better access to extra-hepatic tissues such as Kidney and CNS
- Preclinical data to support the use of these novel delivery systems

David Jackson
Chief Executive Officer
Ceria Therapeutics

6.00 **Chair's Closing Remarks & End of Conference Day One**

> "This summit provides a valuable platform to learn from industry leaders and explore the latest advancements in RNAi therapeutics"
>
> **Chief Executive Officer, SanegeneBio**

> "This event presents an invaluable opportunity to share expertise, gain insights into the latest technical advancements, tackle industry challenges, and engage with leading professionals in the field, fostering both collaboration and innovation"
>
> **Co-founder, Bound Therapeutics**

Conference Day Two
Thursday, January 30, 2025

7.50 Check In & Light Breakfast

Chad Miller
Head of Research
Nosis Bio

8.50 Chair's Opening Remarks

Safeguarding Purity & Quality of Interfering RNA During Large Scale Manufacturing to Enable Sustainable Production of RNAi Therapies

Adam Dinerman
Senior Vice President, Head of CMC
Aro Biotherapeutics
NEW DATA

9.00 Centyrin-siRNA Conjugates: CMC Development Path to Clinic

- Platform manufacturing considerations for Centyrin-siRNA conjugates
- CMC development challenges for protein-oligonucleotide conjugates
- Analytical control strategy considerations and product stability

Dr. Pablo Lores Lareo
CMC Director
Sylentis

9.30 Innovation in Therapeutic-Oligonucleotide Manufacturing to Sustainably Meet Future Demand

- Pursuing a zero-waste strategy that meets Sustainable Development Goals (SDG) in oligonucleotide manufacturing
- Designing and building the future plant: achieving BREEAM certification
- Case study: purification resin substitution

9.40 **Roundtable Discussion:** Streamlining CMC & Maintaining Purity During Scale Up of RNAi Payloads & Delivery Systems to Streamline RNAi Therapeutic Production & Maximize Clinical Success

- Employing analytical methods to measure toxicity and purity of manufactured RNA
- Increasing efficiency during manufacturing and scale up
- How different delivery platforms require differing CMC considerations

Hunghao Chu
Associate Director
Benitec

Adam Dinerman
Senior Vice President, Head of CMC
Aro Biotherapeutics

10.10 Morning Break & Networking

RNAi Therapies in the Clinic: Clinical Data on Innovative Delivery Strategies & Lessons on Maximizing Success of Early-Stage RNAi Assets & Platforms

Zdravka Medarova
Chief Scientific Officer
TransCode Therapeutics
NEW DATA

11.00 Clinical Experience with a First-in-Class ASO Against miR-10b for the Treatment of Metastatic Cancer

- In early discovery efforts, miRNA-10b was identified as a master regulator of the viability of metastatic tumor cells and we developed a therapeutic strategy based on miR-10b inhibition
- The specific inhibition of miR-10b was achieved using ASOs delivered to metastatic sites by TransCode's proprietary delivery system (termed TTX-MC138).
- On the path to clinical development of TTX-MC138, we received FDA authorization for a now completed Phase 0 and an ongoing Phase 1 clinical trial in patients with advanced metastatic cancer

Ashling Holland
Director of Research & Preclinical Development
PepGen
NEW DATA

11.30 Enhanced Delivery Oligonucleotide (EDO) - A Novel Peptide Delivery Platform for Superior Oligonucleotide Delivery

- EDOs are designed to enhance delivery of oligonucleotide therapeutics to muscle and other target tissues
- EDOs show higher uptake to muscle nuclei and corresponds with increased pharmacological activity in multiple preclinical models
- EDO platform has enabled initiation of 2 clinical programs, including a Phase 2 (CONNECT1-EDO51) study to investigate the effects of PGN-EDO51 in people with DMD amendable to exon 51 skipping and a Phase 1 (FREEDOM-DM1) study to investigate the effects of PGN-EDODM1 in people with DM1

Conference Day Two
Thursday, January 30, 2025

12.00 **Interactive Session: Attracting Investment & Securing Preclinical Funding for Early-Stage RNAi Therapeutic Candidates & Platforms**

- How to build a successful network and establish a functioning start up to attract investment
- How to successfully gain funding for preclinical candidates and platform based companies
- What are the hurdles and pitfalls to starting an RNAi therapeutics company and what are the timelines and levels of investment required

Andrew Fraley
Partner
Sunrise Bioventures

Benjamin Kreitman
Principal
Broadview Ventures

Dallece Curley
Associate Director of External Innovation
Eli Lilly

12.45 **Lunch & Networking**

Advancing Cutting-Edge RNAi Therapeutic Delivery Systems to with Increased Selectivity & Durability to Enable Targeting of Hard-To-Drug Tissues

Weimin Wang
Founder & Chief Executive Officer
SanegeneBio

NEW DATA

1.45 **Unlocking the Full Potential of RNAi with LEADTM (Ligand and Enhancer Assisted Delivery)**

- Ligand-mediated conjugate is often insufficient for extrahepatic delivery. Adding an enhancer addresses delivery inefficiencies including PK, receptor binding, internalization, or intracellular trafficking
- Sanegene's innovative Ligand and Enhancer Assisted Delivery (LEAD™) technology has demonstrated robust siRNA delivery and potent gene knockdown across multiple extrahepatic tissues and cells, including muscle, adipose, macrophages, CNS/PNS, and ocular
- Leveraging LEAD™ technology to develop treatments for obesity and metabolic disorders by targeting the liver, muscle, and adipose tissues. LEAD™ technology demonstrates robust gene knockdown, weight loss, and improved body composition in combination with GLP-1RA or siRNA monotherapy

Hanhua Huang
Vice President, Biology
Avidity Biosciences

2.15 **Engineering Antibody Oligonucleotide Conjugates (AOCs): Preclinical, Clinical Progress and Lessons Learned**

- Discussing antibody oligonucleotide conjugates and exploring how AOCs can help solve oligo delivery challenges
- Developing cellular and animal models with relevant disease readouts to demonstrate target engagement;
- AOC development candidate selection and clinical progress

Glenn Kazo
Chief Business Officer
ARIZ Precision Medicine

2.45 **A Ligand-targeted Calcium-phosphate Drug Delivery System for Cancer**

- Alternatives to traditional RNAi delivery approaches are needed for cancer targets
- ARIZ's preclinical novel siRNA formulated in a calcium-phosphate drug delivery system with a tumor targeting ligand, targets a histone methyltransferase that is one of the earliest changes in cancerization of the cell
- Proof of concept with our ligand targeted calcium-phosphate siRNA-conjugate delivery platform broadly solves delivery issues beyond liver cells with specificity for cancer cells.

3.15 **Afternoon Break & Networking**

Conference Day Two
Thursday, January 30, 2025

Overcoming Toxicity & Safeguarding Tissue Specificity with Innovative, Precise Delivery Platforms to Develop Tolerable, Efficacious RNAi Therapies

Ruben Postel
Chief Scientific Officer
Sapreme Technologies

NEW DATA

3.45 **Improving Targeted Endosomal Escape through Sapreme's Endosomal Escape Enhancer Platform for More Potent & Durable Therapeutics**

- Showcasing developable and conjugatable compounds to improve delivery of different RNA payloads through improved endosomal escape
- Showcasing preclinical proof of concept for enhancing endosomal escape through this unique enhancer platform for more potent therapeutics
- Examples how to conjugate this enhancer platform to targeting ligands for extra hepatic delivery

Yuanyuan Jin
Co-founder & Chief Operating Officer
Bound Therapeutics

UNPUBLISHED DATA

4.15 **Targeted Extra-Hepatic Delivery of Custom Oligonucleotide-Peptide Conjugates for Enhanced Tumor Bioavailability, Efficacy, & Safety in a Breast Cancer Model**

- How to design antisense oligonucleotide shortmers with custom RNA amidites for optimal efficacy and reduced off-target effects
- How to enhance tumor uptake and minimize liver accumulation of oligonucleotides via peptide ligand conjugation
- How to design tissue-specific peptide ligands for RNAi therapeutics using AI/ML-driven approach

4.45 **Chair's Closing Remarks & End of 6th RNAi-Based Therapeutics Summit**

> "Sharing cutting edge and novel approaches towards enhancing RNA editing therapeutics to bring drugs to the clinical and to people in need in a safe, robust and timely manner is essential"
>
> **Director of Research and Preclinical Development, PepGen**

Partner With Us

6th Annual RNAi-Based Therapeutics Summit

The **6th RNAi-Based Therapeutics Summit** is the only dedicated forum to unite leaders and decision makers from across the RNAi drug development pipeline looking to progress their novel assets into clinically relevant therapies.

This meeting is your perfect opportunity to connect with the biggest names in the RNAi space to form lasting connections and showcase your innovative solutions. From **R&D support** and **toxicity testing**, to **oligonucleotide synthesis** and **manufacturing**, the RNAi community is seeking resources and guidance to progress the next wave of therapies.

Connect with your target market at this meeting and partner with RNAi leaders, relying on our ability to advertise your brand and establish your company as a top service provider in the field.

Showcase your solutions and excellent customer service

Benefit from pre and post conference exposure and 6+ hours of face-to face networking to share your expertise and service with the hottest prospects

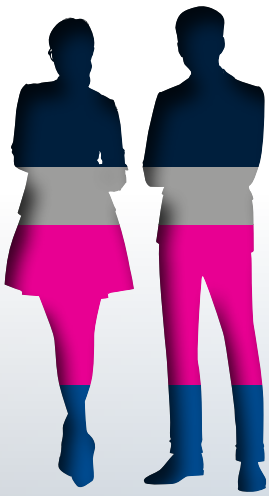
Create lasting impressions

Connect with large pharma leaders from Novo Nordisk and Novartis, and biotech trailblazers from Aro Biotherapeutics, Korro Bio and PepGen, who are seeking cutting edge R&D and manufacturing services

Benefit from market intelligence

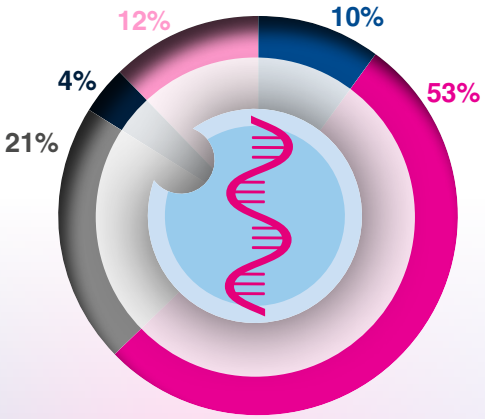
Understand the trends, challenges and ambitions in the RNAi space and gain first-hand insights from decision-makers in the field

SENIORITY OF ATTENDEES*



- C-Level/VP – 33%
- Head/Director – 12%
- Scientist – 33%
- Other – 22%

TYPES OF COMPANIES ATTENDING*



- Academics
- Drug Developer
- Equipment & Service Provider
- Healthcare Provider
- Other

*Based on similar events within the RNA World Series of Events in 2023

GET INVOLVED

Oliver Smare
Partnerships Director
Tel: +1 617 455 4188
Email: sponsor@hansonwade.com

Partner With Us

Innovation Partner

Sylentis offers state-of-the-art GMP-certified facilities for high-quality oligonucleotide production, tailored to clinical trials and commercialization. We provide customized services for small and large-scale synthesis, complex formulations, and specialized synthesis, collaborating closely with clients to meet quality and timeline goals. With extensive expertise in oligonucleotide chemistry and therapeutic applications, Sylentis supports clients through all stages of development. Our flexible manufacturing capacity ensures scalable solutions, while our commitment to quality control, regulatory compliance, and timely delivery at competitive prices fosters strong, collaborative partnerships.

www.sylentis.com

Exhibition Partner

Established in 1981, ChemGenes has consistently provided the highest quality Phosphoramidites and Solid Supports in the market. Our Massachusetts facility is setup for therapeutic grade phosphoramidites for GMP grade oligonucleotide manufacturing.

Furthermore, ChemGenes carries the widest variety of modified phosphoramidites and solid supports currently used in various areas of Nucleic Acid research. ChemGenes remains devoted to providing you with invaluable customer service and comprehensive technical support.

www.chemgenes.com

Exhibition Partner

Headquartered in Morrisville, NC, USA, Synoligo Biotechnologies is a Contract Research and Development Organization (CRDO) specialized in manufacturing complex and highly modified oligonucleotide for academic, biotech start-up and large pharmaceutical communities. Synoligo is a leading oligonucleotide manufacturing company providing high quality, cost-effective custom oligonucleotides at varying scales for research, diagnostic, and therapeutic applications.

www.synoligo.com

Exhibition Partner

Shimadzu Scientific Instruments offers solutions that support RNAi research:

Quadrupole TOF, MALDI-TOF, Bioinert-LC, Compact Mass Detector, Tm Analyzer, LCMS, BioSpec-nano Spectrophotometer, and Data Analysis Software's such as for Oligonucleotide characterization and impurity analysis. Hardware for synthetic chemistry, degradation studies, API delivery to the target tissue, entry into the target cells, reducing off-target results, and studying Phosphorylation RNAi triggers and RISC loading, Genome-wide RNAi screens, as well as Rapid Sequence Confirmation.

For more information please see our link here:

www.ssi.shimadzu.com

GET INVOLVED

Oliver Smare
Partnerships Director
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Email: sponsor@hansonwade.com

Ready to Register?

3 Easy Ways to Book

www.rnaibased-therapeutics.com/take-part/register/

Tel: +1 617 455 4188

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Discover the latest advances in delivery technology and extra-hepatic targeting to unlock the full potential of RNAi therapies to treat a broad range of diseases

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