

December 10-12, 2024 | Boston, MA
www.rna-drugdiscovery.com

REGISTER IN
ADVANCE TO
SAVE

WELCOME

EXPERT SPEAKERS

AGENDA

PARTNER WITH US

REGISTER YOUR PLACE



7th Annual
**RNA-Targeted Drug Discovery
& Development Summit**

Supercharging the Translation of RNA Targeted Therapies Towards the Clinic

**Optimizing Lead Discovery, Structural
Analysis & Preclinical Modeling to
Successfully Develop Stable, Effective &
Orally Bioavailable Small Molecule RNA
Drugs for Robust Translation into the Clinic**

Expert Speakers Include:



Jennifer Petter
Founder & Chief
Scientific Officer
**Arrakis
Therapeutics**



Chris Yates
Executive Director,
Head, Medicinal
Chemistry
**Rgenta
Therapeutics**



Perla Breccia
Senior Director,
Chemistry
AstraZeneca



Anu Bhattacharyya
Vice President,
Splicing Drug
Development
PTC Therapeutics



Joon Lee
Associate Scientific
Director
Biogen



Helen Crehan
Principal Scientist,
Pharmacology
**Expansion
Therapeutics**

Proud to Partner With:



+1 617 455 4188 @ info@hansonwade.com

www.rna-drugdiscovery.com World RNA Series



Welcome to the 7th RNA-Targeted Drug Discovery & Development Summit

Continuing to break new ground and make significant progress, the RNA-targeting field is progressing towards improved selectivity, stability, and RNA conformational dynamics. In 2024, we have already seen **Rgenta Therapeutics receive FDA approval on their IND filing**. It is clear this community is continuing to thrive with innovative therapeutic approaches for previously undruggable RNA targets.

The **7th RNA-Targeted Drug Discovery & Development Summit** returns as the **only and most comprehensive forum** for the RNA-targeting small molecule community to unite. RNA stakeholders will streamline the route of RNA candidates from preclinic into clinical development, bringing effective drugs to patients faster by **advancing the understanding of the relationship between RNA structure and function, deciphering conformational dynamics, and designing small molecules that can effectively bind and stabilize RNA structures**.

Join expert minds from **Arrakis Therapeutics, Ribometrix, Expansion Therapeutics, PTC Therapeutics** and **Remix Therapeutics**, to hear first-hand data-driven presentations to accelerate lead discovery, preclinical translation and clinical approval. As **advances in high-throughput screening, computational modeling, and RNA structural analysis** are happening, this meeting is your prime opportunity to share, learn and collaborate with industry leaders to advance the field towards gaining robust clinically validated RNA small molecule candidates.

Bringing together 100+ biopharma and academic experts in **RNA Science, Chemistry, Structural Biology, Biophysics and Medicinal Chemistry**, join us to access your ultimate roadmap to successfully **fast-track your drugs from early discovery concept to proven clinical candidates, and realize the full potential of this promising field for patients in need**.

What you can expect ...



100+
Attendees



25+
World-Class Speakers



16+
Data-driven Presentations



7+
Hours of Dedicated Networking



3
Deep Dive Workshops



1
Exclusive Focus Day

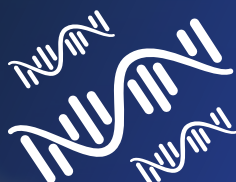


1
Scientific Poster Session

KEY BENEFITS OF ATTENDING



DE-RISK translation of RNA-targeting drugs through improving preclinical modeling strategies and toxicity testing for more accurate and reliable safety profiling with **PTC Therapeutics, Expansion Therapeutics & Sanofi**



DEEP DIVE into RNA target structures to identify disease-relevant RNAs and optimize small molecule selectivity for more efficacious therapeutics with **Base4 Biotechnology, Ribometrix & Arrakis Therapeutics**



REVOLUTIONIZE the treatment of neurological and oncological indications through optimizing selectivity of orally bioavailable RNA-targeting small molecules with **PTC Therapeutics, Rgenta Therapeutics, ReviR Therapeutics & University of Toronto**



ILLUMINATE novel targets beyond splicing, such as lncRNAs and repeat RNAs, to expand the potential of the RNA targeting field, enabling treatment of a wider landscape of disease with **AstraZeneca & Massachusetts Institute of Technology**



LEVERAGE proteins and RNA-protein complexes to target undruggable disease causing RNA and drive more potent therapeutics towards the clinic with **Remix Therapeutics & Accent Therapeutics**

What's New for 2024?

7th Annual
RNA-Targeted Drug Discovery
& Development Summit

December 10-12, 2024 | Boston, MA

WELCOME

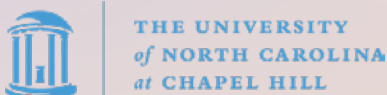
EXPERT SPEAKERS

AGENDA

PARTNER WITH US

REGISTER YOUR PLACE

Companies Sharing Brand New Data



New & Noteworthy Sessions Include



Utilizing RNA-Seq Data At-Scale to Improve the Translatability of mRNA Splicing Modulators

Steve Lianoglou, Director, Translational Genomics, **ReviR Therapeutics**

Large-Scale Discovery of RNA Tertiary Structures & Disease Relevant RNA Targets

Kevin Weeks, Kenan Distinguished Professor, **University of North Carolina at Chapel Hill**



Showcasing Insights to A-Bulge Splice Modulator Selectivity Derived from Studies of Minigenes & Other Genetic Instruments

Joon Lee, Associate Scientific Director, **Biogen**

Leveraging Strategies to Drug RNA-Protein Complexes with Small Molecules to Drive More Novel Therapeutics Towards the Clinic

Dominic Reynolds, Chief Scientific Officer, **Remix Therapeutics**



Panel Discussion - Discussing Investment in RNA-Targeting Therapeutics to Accelerate Advancement of Small Molecule Therapies for Patients in Need

Joy Ghosh, Senior Portfolio Manager & Head, Life Sciences, TFG Asset Management, **Tetragon Financial Management**

Yang Zheng, Head, Business Development, **Base4 Biotechnology**

David Brower, Chief Business Officer, **Accent Therapeutics**

Raymond Kelleher, Managing Director, **Cormorant Asset Management**

Jennifer Petter, Founder & Chief Scientific Officer, **Arrakis Therapeutics**

Showcase Your Scientific Poster



Contribute to the conversation and share your cutting-edge research with your fellow RNA-targeting community to showcase your breakthrough discoveries to a vast audience of experts.

Register your place and submit an abstract for review to showcase your poster*

*Please visit the website for the Ts&Cs for presenting a poster

Agenda at a Glance

Pre-Conference Focus Day & Workshop Day: Tuesday, December 10

Safety & Off-Target Toxicity Profiling Focus Day	Workshop Day
Rethinking Safety Testing for Small Molecules Targeting RNA to Improve Understanding of Off-Target Effects for Reduced Toxicity	Workshop A: Leveraging Innovative Computational & High Throughput Screening Approaches to Accurately Identify Select RNA Binders for Improved Therapeutics Effects
Morning Networking Break	
Accomplishing Successful Translation Through Improved Preclinical Model Selection, Off-Target Understanding & Safety Testing to Advance Safe & Potent Small Molecules	Workshop B: Achieving Good Small Molecule Drugs by Delving into Optimal Chemical Features to Fast-Track Hit Discovery of Relevant Small Molecules
Lunch & Networking Break	
Leveraging Strategies to Improve Human RNA Representation in Preclinical Models for more Reliable Translation of Small Molecule Drugs	Workshop C: Uncovering RNA Targets Beyond Splicing Modifiers to Expand the Potential of RNA Targeting for Treating More Diseases
End of Pre-Conference Focus & Workshop Day	

Conference Day One: Wednesday, December 11

Improving Target Prediction by Supercharging RNA Structure Understand & Target Discovery Using Primary, Secondary & Tertiary Structure Data
Morning Break & Speed Networking
Exploiting Strategies for Accelerating Disease Relevant RNA Target Identification to Ensure Functional Outcomes
Lunch & Networking Break
Fast-Tracking Potent Lead Candidates Towards Patients by Improving Discovery & Characterization of Drug-Like Small Molecules
Afternoon Networking Break & Scientific Poster Session
Uncovering Novel Targets on Proteins, Enzymes & RNA/Protein Complexes to Improve Targeting of Disease-Causing RNA for More Efficacious Treatments
End of Conference Day One

Conference Day Two: Thursday, December 12

Advancing Specific & Efficacious Small Molecules Targeting RNA for Treating Neurological Disorders to Help the Patients in Need
Morning Networking Break
Unravelling the Biology of Splicing to Advance Small Molecule Modulators & Identify Novel Splice Sites to Widen the Landscape of Treatable Diseases
Lunch & Networking Break
Unlocking Small Molecule's Potential in the RNA-Targeting Field to Increase Investment & Treatment of a Wider Portfolio of Disease
Afternoon Networking Break - Energize & Engage!
Optimizing Small Molecule Design for Greater Selectivity & Binding Affinity to Advance More Effective RNA Targeting Therapeutics & Imaging Agents
End of the 7th RNA-Targeted Drug Discovery & Development Summit 2024

Your Expert Speakers



Kenneth Duncan
Senior Vice President,
Chemistry
Accent Therapeutics



David Brower
Chief Business Officer
Accent Therapeutics



Domi Stickens
Vice President, Biology &
Translational Sciences
Arrakis Therapeutics



Perla Breccia
Senior Director, Chemistry
AstraZeneca



Rhona Cox
Director, Medicinal
Chemistry
AstraZeneca



Yang Zheng
Head, Business
Development
Base4 Biotechnology



Hashim Al-Hashimi
Co-Founder & Platform
Architect
Base4 Biotechnology



Joon Lee
Associate Scientific
Director
Biogen



Helen Crehan
Principal Scientist,
Pharmacology
Expansion Therapeutics



Peter Connolly
Vice President, Structural
Biology & Biophysics
Expansion Therapeutics



Chris Burge
Professor
**Massachusetts Institute
of Technology**



Vipender Singh
Senior Principal Scientist
& Project Team Leader
**Novartis - Biomedical
Research**



Tom Chappie
Research Fellow
Pfizer



Kerstin Effenberger
Principal Scientist,
Discovery Splicing
PTC Therapeutics



Anu Bhattacharyya
Vice President, Splicing
Drug Development
PTC Therapeutics



Dominic Reynolds
Chief Scientific Officer
Remix Therapeutics



Paul August
Chief Scientific Officer
ReviR Therapeutics



Steve Lianoglou
Director, Translational
Genomics
ReviR Therapeutics



Chris Yates
Executive Director, Head,
Medicinal Chemistry
Rgenta Therapeutics



Matt Friedersdorf
Associate Director,
Translation Medicine
Ribometrix



Yann Foricher
Head, Small Molecules
Medicinal Chemistry
Sanofi

Your Expert Speakers



Joy Ghosh
Senior Portfolio Manager &
Head, Life Sciences, TFG
Asset Management
**Tetragon Financial
Management**



Kevin Weeks
Kenan Distinguished
Professor, Chemistry
**University of North
Carolina at Chapel Hill**



Amanda Hargrove
Professor, Chemistry
University of Toronto



John Schneekloth
Senior Investigator
National Cancer Institute



Chuhern Hwang
Co-Founder & Head, Drug
Discovery
Wayfinder Biosciences



Shunnichi Kashida
Co-Founder,
Representative Director,
President & Chief
Executive Officer
xFOREST Therapeutics



Raymond Kelleher
Managing Director
**Cormorant Asset
Management**



Jennifer Petter
Founder & Chief Scientific
Officer
Arrakis Therapeutics



Yan Song
Senior Principal Scientist
Amgen



Sichong Peng
Bioinformatics Scientist
Eclipsebio



Karen Wu
Co-Founder & President
Lucerna, Inc.



Can Ozbal
Founder & Chief Executive
Officer
**Momentum
Biotechnologies**

“ This conference in the past 6 summits presented some of the most interesting and state of the art developments in targeting RNA and RNA modulating enzymes. I expect to be enthralled again with the deep discussions from the delegates and look forward to being part of this group ”

**Kenneth Duncan, Senior Vice President, Chemistry,
Accent Therapeutics**

Safety & Off-Target Toxicity Profiling Focus Day

Tuesday, December 10

Join us for a dedicated day on key challenges including safety and off target toxicity that are currently holding back the field of RNA-targeting small molecules field. Through evaluating reliable preclinical models, safety margin considerations and off-target pharmacology, this day will provide you with actionable insights to fast-track successful translation of your preclinical pipelines into robust clinical candidates with higher rate of success.



8.00 **Check-in Opens & Light Breakfast**



Yan Song
Senior Principal Scientist
Amgen

9.05 **Chair's Opening Remarks**

Rethinking Safety Testing for Small Molecules Targeting RNA to Improve Understanding of Off-Target Effects for Reduced Toxicity

9.15 **Evaluating Safety & Toxicity Packages for Marketed & Late-Stage Clinical Investigational Drugs**



Helen Crehan
Principal Scientist, Pharmacology
Expansion Therapeutics

- Highlighting the safety and toxicity packages which are made to advance safe small molecules targeting RNA towards the patients in need
- Evaluating the safety window which is required for regulatory approval to fast-track RNA-targeting therapeutics towards the clinic
- Uncovering reliable preclinical models used to represent RNA targets and analyse small molecule safety

9.30 **Roundtable Discussion: Evaluating Regulators' Views on Safety & Toxicity Testing to Streamline Preclinical Testing & Fast-Track RNA-Targeting Drug Approval**



- What are regulators' thoughts on using animal models to showcase off-target effects and toxicity given their limited RNA conservation?
- What safety and toxicity tests are required to gain FDA approval for advancing into human clinical trials?
- Highlight specifications which are required to be met by RNA-targeting drugs to ensure regulatory approval.



Anu Bhattacharyya
Vice President, Splicing Drug Development
PTC Therapeutics



Helen Crehan
Principal Scientist, Pharmacology
Expansion Therapeutics



10.30 **Morning Break & Networking**

Accomplishing Successful Translation Through Improved Preclinical Model Selection, Off-Target Understanding & Safety Testing to Advance Safe & Potent Small Molecules

11.30 **Utilizing RNA-Seq Data At-Scale to Improve the Translatability of mRNA Splicing Modulators**



Steve Lianoglou
Director, Translational Genomics
ReviR Therapeutics

NEW DATA

- Outlining the unique translational challenges in developing small molecule modulators of mRNA splicing
- Summarizing use of multi-dose RNA-seq experiments to identify potentially toxic off target liabilities for development candidates
- Summarizing use of multi-species RNA-seq data to improve animal model selection during preclinical research



Vipender Singh
Senior Principal Scientist & Project Team Leader
Novartis - Biomedical Research

12.00 **Targeting RNA Genomes with Small Molecules to Develop Antiviral Therapeutics**

- Targeting or mutagenesis or evolution for developing antiviral therapeutics
- Safety considerations to avoid mutagenesis to host genomes
- Improving efficacy and safety of the antiviral therapeutics using an analytical platform

Safety & Off-Target Toxicity Profiling Focus Day

Tuesday, December 10



John Schneekloth
Senior Investigator
National Cancer Institute

12.30

NEW DATA

NEW COMPANY

Investigating RNA Off-Target Effects of FDA Approved Drugs to Understand Pharmacology

- Small molecule screening reveals many drug-like compounds bind to RNA
- Using AI/ML to understand that many FDA approved drugs have RNA binding potential
- Transcriptome-wide binding and functional analysis reveals the impact of RNA binding on off-target pharmacology



1.00 Lunch Break & Networking

Leveraging Strategies to Improve Human RNA Representation in Preclinical Models for More Reliable Translation of Small Molecule Drugs

2.00 Expert Led Breakout Session: Translating an RNA-Targeting Small Molecule from a Hit to a Human Using Novel Preclinical Modeling Strategies to Fast-Track Safe Therapeutics Towards Patients

A major challenge for the translation of safe RNA targeting therapeutics is understanding RNA conservation across different animal models. Disease causing RNA sequences can change from animal to animal, therefore, new and innovative approaches need to be developed to enable reliable pre-clinical testing for accelerating safe and efficacious therapeutics towards the patients in need.

During this interactive session, join forces with your peers to discuss different strategies for overcoming this challenge as well as uncovering novel and innovative approaches to preclinical modeling. Here are some questions to think about during this session:

- Are there ways to manipulate animal models to improve their representation of human RNA?
- Which models and screening methods are used to successfully translate a small molecule drug from hit discovery and into the clinic?
- How to ensure the animal models are representative of the mechanism which is occurring in humans?
- What new technologies have been developed to minimize the need to use animal models and more accurately represent humans?
- What are the opportunities and challenges of organoid and organ-on-chip models for advancing RNA-targeting drugs?



Yann Foricher
Head of Small Molecules Medicinal Chemistry
Sanofi



Steve Lianoglou
Director, Translational Genomics
ReviR Therapeutics



Yan Song
Senior Principal Scientist
Amgen

3.30 Chair's Closing Remarks & End of Pre-Conference Focus Day

▀▀ I believe this meeting will inspire new ideas and methodologies that I can bring back to my lab, further advancing our research in RNA therapeutics. ▀▀

Alisha Jones, Assistant Professor, Chemistry, New York University

Pre-Conference Workshop Day

Tuesday, December 10

Check-in Opens & Light Breakfast

8.00

Workshop A

9.00

Leveraging Innovative Computational & High Throughput Screening Approaches to Accurately Identify Selective RNA Binders for Improved Therapeutic Effects

To carefully select the most promising small molecules and RNA targets, many new and innovative high throughput screening and computational based approaches have been developed. However, the field is currently plagued by limited sensitive and robust methods, hence, in this session the community will uncover latest strategies for identifying selective and specific small molecules.

This session will focus on:

- Deep diving into novel computational models and screening strategies to identify disease-relevant RNA targets and selective RNA binders
- Improving synergy between different computational and screening strategies to accelerate RNA target discovery
- Uncovering new methodologies which have been developed which are more robust and reproducible

Workshop Leaders



Chuhern Hwang
Co-Founder
& Head, Drug
Discovery
**Wayfinder
Biosciences**



Shunnichi Kashida
Co-Founder,
Representative
Director, President
& Chief Executive
Officer
**xFOREST
Therapeutics**

Morning Break & Networking

11.00

Workshop B

12.00

Achieving Good Small Molecule Drugs by Delving into Optimal Chemical Features to Fast-Track Hit Discovery of Relevant Small Molecules

There's currently only a small variety of small molecules which have bound to RNA and shown functional effects. Hence, there's only a small number of relevant small molecule drugs to compare your drug discovery too. To ensure you are advancing the right small molecules, it is vital to understand what pharmacological features to look out for to ensure they can be developed into drugs later in the drug development pipeline.

This session will focus on:

- What is the pharmacology of a good small molecule drug targeting RNA?
- Identifying chemical features which make small molecules good drugs, such as understanding the ideal permeability and stickiness
- Understanding the key pharmacological properties which small molecule drugs need to enable optimization of algorithms and models
- How does a good small molecule interact with the RNA target, both in a cell and in a disease setting?

Workshop Leaders



Perla Breccia
Senior Director,
Chemistry
AstraZeneca



Tom Chappie
Research Fellow
Pfizer

Lunch & Networking

2.00

Pre-Conference Workshop Day

Tuesday, December 10

Workshop C

3.00

Uncovering RNA Targets Beyond Splicing Modifiers to Expand the Potential of RNA Targeting for Treating More Diseases

There are many novel RNA targets in addition to splice sites, such as long non-coding RNAs which have highly structured motifs, meaning they are good targets. Hence, understanding the new and innovative areas to expand this field is exciting and can increase the potential of treating more diseases.

This session will focus on:

- Uncovering the involvement of long non-coding RNAs in disease and understanding if they are suitable to target by small molecules
- Identifying new disease relevant RNA sequences, beyond splice sites
- Highlighting RNA targets for ASOs which may be relevant for targeting by small molecule drugs for better oral bioavailability
- How can targeting repeat RNAs lead to functional consequences?

Workshop Leaders



Rhona Cox
Director, Medicinal
Chemistry
AstraZeneca



Chris Burge
Professor
**Massachusetts
Institute of
Technology**



End of Pre-Conference Workshop Day

5.00

▀▀ This meeting provides invaluable opportunities for collaboration, knowledge exchange, and innovation. Engaging with other experts and industry leaders fosters a dynamic environment where new ideas can be explored, and current challenges addressed collectively. The value lies in the ability to contribute to and learn from cutting-edge research, gain diverse perspectives, and identify potential partnerships that can accelerate therapeutic development. ▀▀

Paul August, Chief Scientific Officer, ReviR Therapeutics

Conference Day One

Wednesday, December 11



8.30 Check-in Opens & Light Breakfast



Rhona Cox
Director, Medicinal
Chemistry
AstraZeneca

9.20 Chair's Opening Remarks

Improving Target Prediction by Supercharging RNA Structure Understand & Target Discovery Using Primary, Secondary & Tertiary Structure Data



Hashim Al-Hashimi
Co-Founder & Platform
Architect
Base4 Biotechnology

9.30

Keynote Address: Unlocking RNA Dynamics for Targeted Drug Discovery

- Unraveling the intricate nature of RNA dynamics to drive rational drug discovery
- Leveraging RNA dynamics to reveal novel mechanisms of splicing and gene expression modulation
- Dynamics is key to translating RNA/SM interactions into actionable SAR insights



Kevin Weeks
Kenan Distinguished
Professor, Chemistry
University of North
Carolina at Chapel
Hill

10.00

Large-Scale Discovery of RNA Tertiary Structures & Disease Relevant RNA Targets

- RNA motifs with complex 3D structures are promising targets for small molecule therapeutics but are still very hard to find
- A new technology that identifies RNA tertiary structures, transcriptome-wide, typically with direct functional roles, will be presented
- Thousands of targetable sites likely exist across the human transcriptome



Sichong Peng
Bioinformatics Scientist
Eclipsebio

10.30

Multidimensional Profiling & Validation of Small Molecule Targets

- Discover multiomic approaches for target discovery and validation
- Identify disease-specific, druggable pockets
- Validate small molecule binding to target RNAs



10.40 Morning Break & Speed Networking

This session is your opportunity to get face-to-face with many of the brightest minds working in the RNA targeting small molecule field, and establish meaningful business relationships to pursue for the rest of the conference.

Exploiting Strategies for Accelerating Disease Relevant RNA Target Identification to Ensure Functional Outcomes



Peter Connolly
Vice President,
Structural Biology &
Biophysics
Expansion
Therapeutics

11.30

NMR as a Tool for Structure Characterization of RNA Targets in Drug Discovery

- Using methods to predict and confirm RNA secondary structure
- Using NMR to better understand the structure of RNA in structure guided drug design
- Using NMR in combination with other methods to characterize drug binding on RNA targets



Karen Wu
Co-Founder &
President
Lucerna, Inc.

12.00

New Fluorogenic Toolkits for Diverse RNA-Targeted Drug Discovery Applications

- Real-time imaging tools to track RNA movement, measure RNA half-life, and assess RNA target engagement in cells
- HTS platforms for RNA degradation, splicing, aggregation, and structure-focused hit identification

Conference Day One

Wednesday, December 11

12.10 Panel Discussion: Evaluating High-Value Direct RNA & Protein/RNA Complex Target Traits for Ensuring Efficient Binding & Therapeutic Effect for Accelerating Relevant Drug Discovery



- What are the key characteristics which validated RNA and RNA/protein targets share?
- How to conduct high resolution characterization of RNA targets including the different proteins and binding compounds present in the body
- How to determine RNA sub-targets which can be both bound by small molecule targets and cause therapeutic effects



Dominic Reynolds
Chief Scientific Officer
Remix Therapeutics



Domi Stickens
Vice President, Biology &
Translational Sciences
Arrakis Therapeutics



Paul August
Chief Scientific Officer
ReviR Therapeutics



Matt Friedersdorf
Associate Director,
Translation Medicine
Ribometrix



1.00 Lunch & Networking

Fast-Tracking Potent Lead Candidates Towards Patients by Improving Discovery & Characterization of Drug-Like Small Molecules

2.00 Genetic Medicines: Discovering Small Molecules that Interdict RNA Function



Domi Stickens
Vice President,
Biology & Translational
Sciences
Arrakis Therapeutics

NEW
DATA

- RNA targets are now broadly validated clinically and commercially, but many attractive targets remain out of reach to current modalities
- Arrakis has built a novel and highly differentiated platform for discovering drug-like small molecule ligands for RNA targets
- Arrakis platform has thus far produced at least two small molecule programs that clearly mimic the biological activity of oligonucleotide therapeutics

2.30 A Complete Workflow for the Identification & Characterization of Small Molecule Modulators of RNA



Can Ozbal
Founder & Chief
Executive Officer
Momentum
Biotechnologies

- A comprehensive workflow starting with approaches to identify small molecules that selectively bind an RNA of interest, determining the binding affinity of binders to target and off-target sequences, and characterization of the functional activity of those binders in cell-based proteomics assays will be presented
- Additional workflows to quantify and characterize covalent/irreversible adducts of RNA will be also be discussed



3.00 Afternoon Networking Break & Scientific Poster Session

Contribute to the conversation and share your cutting-edge research with your fellow RNA-targeting community to showcase your breakthrough discoveries to a vast audience of experts. Register your place to submit an abstract for review to showcase your poster*

*Please visit the website for the T&Cs for presenting a poster

Uncovering Novel Targets on Proteins, Enzymes & RNA/Protein Complexes to Improve Targeting of Disease-Causing RNA for More Efficacious Treatments

4.00 Leveraging Strategies to Drug RNA-Protein Complexes with Small Molecules



Dominic Reynolds
Chief Scientific Office
Remix Therapeutics

- Identifying disease relevant RNA-protein targets for previously intractable diseases
- Developing screening assay strategies for the discovery of novel small molecule splicing modulators
- Optimizing toward more potent and efficacious small molecule therapeutics: moving from early discovery to preclinical development for more streamlined translation towards the clinic

Conference Day One

Wednesday, December 11



Kenneth Duncan
Senior Vice President,
Chemistry
Accent Therapeutics

4.30



RNA-Modifying Enzyme Inhibitors as Synthetic Lethal Cancer Therapeutics

- Expanding the potential impact of helicase inhibitors beyond MSI-H cancers with DHX9-targeted therapeutics
- Validation and drug discovery enablement of RNA modifying nuclease target XRN1 in tumors with elevated interferon signaling



Rhona Cox
Director, Medicinal
Chemistry
AstraZeneca

5.00

Chair's Closing Remarks & End of Conference Day One

It will be a great opportunity to share and learn from experiences across different companies.

Rhona Cox, Director, Medicinal Chemistry, AstraZeneca

I look forward to discussing the latest findings with industry and academic colleagues and learning about new therapeutic approaches.

**Chris Burge, Professor,
Massachusetts Institute of Technology**

Conference Day Two

Thursday, December 12



8.20 Check-In Opens & Light Breakfast



Kenneth Duncan
Senior Vice President,
Chemistry
Accent Therapeutics

8.50 Chair's Opening Remarks & Common Interests Icebreaker

Advancing Specific & Efficacious Small Molecules Targeting RNA for Treating Neurological Disorders to Help the Patients in Need



Chris Yates
Executive Director,
Head, Medicinal
Chemistry
Rgenta Therapeutics

9.00 **Developing RNA-Modulating Oral Small Molecules Targeting PMS1 to Treat Incurable Neurological Disorders**



- Leveraging our proprietary, integrative RNA-targeting small molecule discovery platform to pioneer the development of first-in-class oral therapies
- Pursuing oncology and neurological disease targets, exemplified by the oncogenic transcription factor target c-MYB and the PMS1 gene
- PMS1 is a key component of the DNA mismatch repair pathway, implicated in the pathological somatic trinucleotide repeat expansion observed in Huntington's Disease (HD) and other trinucleotide repeat expansion disorders



Anu Bhattacharyya
Vice President, Splicing
Drug Development
PTC Therapeutics

9.30 **PTC518 Mediated Splice-Induced HTT Lowering: Pathway from Concept to Patients**

- Uncovering how animal models demonstrated that reducing mutant huntingtin protein (mHTT) levels could alleviate Huntington's disease (HD)-like symptoms
- Reviewing the discovery and development of PTC518, a small molecule splicing modifier that reduces the production of the mHTT protein in cells and animals
- Highlighting PTC518's clinical data to demonstrate proof of mechanism and dose-dependent lowering of HTT mRNA and protein levels in healthy volunteers and HD patients



10.00 Morning Networking Break

Unravelling the Biology of Splicing to Advance Small Molecule Modulators & Identify Novel Splice Sites to Widen the Landscape of Treatable Diseases



Chris Burge
Professor
Massachusetts
Institute of
Technology

11.00 **Splicing: Predictive Models & Variability Between People**



- KATMAP learns a regulatory activity map for splicing factors using knockdown and *in vitro* RNA-binding data
- Direct regulatory target exons and associated regulatory elements are predicted
- Using GTEx RNA-seq data, we have identified tens of thousands of "naturally variable exons" (NVEs) that are spliced in some people but not others and shown that NVEs aid in interpretation of genetic variants



Joon Lee
Associate Scientific
Director
Biogen

11.30 **Showcasing Insights to A-Bulge Splice Modulator Selectivity Derived from Studies of Minigenes & Other Genetic Instruments**



- Uncovering effects of splice site modification on splice modulator activity
- Delving into transcriptomic comparisons of different splice modulator modalities to identify selective compounds
- Deep diving into highly-specific splice modulator discovery to advance development of small molecules



Kerstin Effenberger
Principal Scientist,
Discovery Splicing
PTC Therapeutics

12.00 **Identifying Small Molecules Targeting Novel Splice Sites Leading to Therapeutic Effect**



- Implementation of PTSeekTM to discover novel small molecules that target splice sites found in intron-derived exons of therapeutically relevant targets
- Advancement of splicing modifiers to treat human diseases of unmet medical need

Conference Day Two

Thursday, December 12

12.30 **Unlocking the Therapeutic Potential of Small Molecule mRNA Splicing Modulators to Prevent Somatic Expansion in Huntington's Disease**



Paul August
Chief Scientific Officer
ReviR Therapeutics



- Exploring the molecular mechanisms by which small molecule mRNA splicing modulators interact with the mismatch repair (MMR) pathway to prevent somatic expansion
- Summarizing results from *in vivo* studies that evaluate the therapeutic potential of small molecule mRNA splicing modulators to address somatic expansion
- Highlighting key findings that show the impact on the mismatch repair pathway, including changes in gene expression, protein levels, and cellular phenotypes relevant to Huntington's Disease



1.00 **Lunch & Networking**

Unlocking Small Molecule's Potential in the RNA-Targeting Field to Increase Investment & Treatment of a Wider Portfolio of Disease

2.00 **Panel Discussion: Discussing Investment in RNA-Targeting Therapeutics to Accelerate Advancement of Small Molecule Therapies for Patients in Need**



- What motivation do venture capitalists need to invest in RNA targeting small molecule therapeutics?
- What do venture capitalists view as critical points of investment both in preclinical and early clinical development?
- What do investors look for in investable ideas and approaches within the RNA-targeting field?



Joy Ghosh
Senior Portfolio Manager &
Head, Life Sciences, TFG Asset
Management
Tetragon Financial Management



Yang Zheng
Head, Business Development
Base4 Biotechnology



David Brower
Chief Business Officer
Accent Therapeutics



Raymond Kelleher
Managing Director
Cormorant Asset Management



Jennifer Petter
Founder & Chief Scientific Officer
Arrakis Therapeutics



3.00 **Afternoon Networking Break - Energize & Engage!**

Network with your peers aside our beneficial light therapy lamps to rejuvenate before the last few sessions

Optimizing Small Molecule Design for Greater Selectivity & Binding Affinity to Advance More Effective RNA Targeting Therapeutics & Imaging Agents

3.30 **Small Molecules Modulate Stability of MALAT1 Substructures**



Amanda Hargrove
Professor, Chemistry
University of Toronto



- RNA-targeted libraries yield small molecule modulators of RNA conformation and function
- Oncogenic MALAT1 lncRNA contains ligandable substructures with putative function
- Tunable small molecules modulate MALAT1: protein interactions and lead to rapid degradation



John Schneekloth
Senior Investigator
National Cancer
Institute



4.00 **Structure- & Fragment- Based Design of Small Molecule RNA Imaging Agents**

- Fluorogenic RNA aptamers are powerful tools to image RNA in live cells
- Broad adaptation of this technology requires new, brighter fluorophore systems
- Structure- and fragment- based design enables the discovery of an ultrabright RNA activated fluorophore



Kenneth Duncan
Senior Vice President,
Chemistry
Accent Therapeutics

4.30 **Chairs Closing Remarks**

4.45 **End of 7th RNA-Targeted Drug Discovery & Development Summit 2024**



Program Partner

Momentum is a specialized CRO focused on providing mass spectrometry-based native detection technologies for lead discovery and the quantification of difficult targets to biopharmaceutical clients.

www.momentum.bio



Innovation Partner

Eclipsebio is a biotech company headquartered in San Diego, developing first-in-class assays and analytical solutions to accelerate the development of tomorrow's RNA medicines. The company offers its end-to-end RNA capabilities as collaborative partnerships and as end-to-end services.

www.eclipsebio.com



Innovation Partner

Lucerna, Inc. is a RNA-focused tool company focused on developing and commercializing the fluorescent aptamer technology (Spinach™) for the purposes of: 1.) Providing plug-and-play RNA imaging and detection systems as research reagents, 2.) Developing high-throughput screening platforms to enable new drug discovery of previous intractable targets, and 3.) Developing fluorescent sensors for industrial production applications.

www.lucernatechnologies.com



Exhibition Partner

We share your passion for science. Promega Corporation is a world leader in providing innovative solutions for the life sciences. We develop bioluminescent technologies that deliver more biologically relevant data for small and large molecule drug discovery. Our broad life science portfolio includes biochemical and cell-based assays for drug discovery enabling research focused on hot targets like kinases, DDR/PARP, and RAS, and innovative modalities like induced proximity and targeting RNA. Let's collaborate.

www.promega.com



Event Partner

Maratech NMR Solutions is a life sciences partner located in Cambridge Massachusetts specializes in state of the art Nuclear Magnetic Resonance Spectroscopy. We offer a wide range of services including both experimental and consulting support. Our scientists have over 24 years experience in the pharmaceutical industry advancing drug discovery research through the use of NMR. We work with our partners by utilizing our NMR technology to rapidly and efficiently deconvolute complex chemical and biological interactions that are often bottlenecks for research. Maratech NMR Solutions provides support for organizations that have complex research problems where NMR spectroscopy may be of value or where high-level strategic input is required.

www.maratechnmr.com



Event Partner

With the ever-growing demand for large-scale gene expression data, Alithea Genomics' innovative technologies streamline the sequencing workflow by enabling the simultaneous preparation of thousands of samples in a massively multiplexed format. This drastically reduces time, cost, and labor, allowing researchers to focus on data analysis and discovery. Our latest advancements include the capability to prepare libraries for both full-length mRNA sequencing and 3' mRNA-seq utilizing DRUG-seq, our RNA-extraction free solution, providing unparalleled flexibility in transcriptome analysis for drug discovery.

www.alitheagenomics.com

Partner With Us

The RNA targeting field is rapidly evolving with drug developers around the world racing to develop best-in-class small molecules. However, to continue accelerating this community through discovery and towards the clinic, leading experts are looking for service providers with capabilities in **genomic profiling, biophysical screening, assay development, RNA sequencing, discovery and preclinical services, NMR services and computational modeling** to support their RNA target structure understanding as well as improve their preclinical safety and efficacy studies.

Therefore, the **7th RNA Targeted Drug Discovery & Development Summit** is returning to Boston as your premium opportunity to meet and network with decision makers from RNA-targeting biopharma, including experts from the likes of **Biogen, AstraZeneca, Pfizer, PTC Therapeutics, Remix Therapeutics** and more, maximizing your opportunity to generate revenue and long-lasting connections.



Benefit From Market Intelligence

With many new RNA-targeting approaches being developed, from splicing modulation to targeting long non-coding RNAs, hear how and where biopharma are searching for services and solutions to facilitate their RNA targeted drug discovery and match your solutions accordingly



Meet & Network In-Person with Industry Pioneers

With a room full of drug developers from the likes of Pfizer, Biogen, AstraZeneca, Sanofi, Expansion Therapeutics, Ribometrix and more, meet prospective clients during speed networking breaks and informal networking receptions to fuel commercial opportunities.



Position Yourself as an Industry Expert

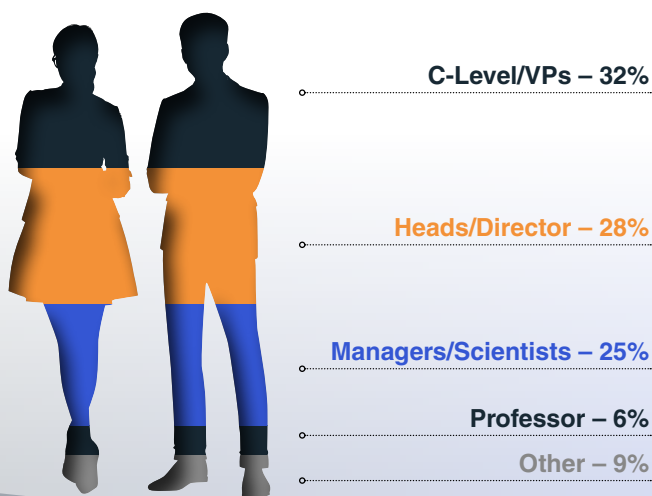
With the growing landscape of RNA targeting biotech companies, this meeting is a dedicated platform to put your independent expertise in front of the key decision-makers in the field.



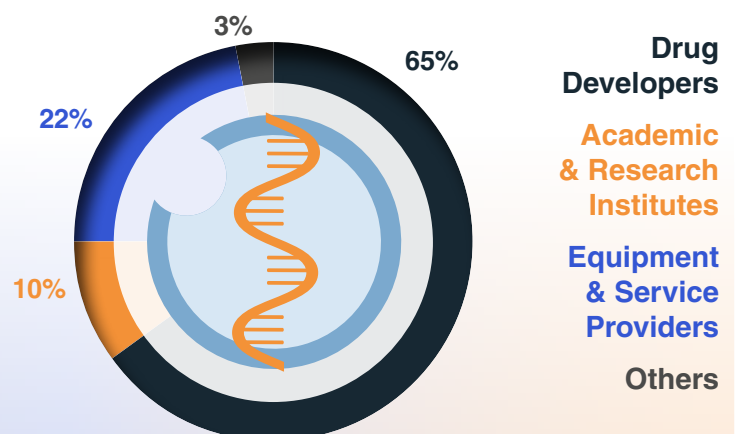
Raise Brand Awareness & Generate Commercial Collaborations

Benefit from pre- and post-conference exposure to our comprehensive RNA-small molecules community as well as provide a wish-list of your choice to be contacted in advance to ensure you are set up for success at this valuable and dedicated meeting

SENIORITY OF ATTENDEES*



TYPES OF COMPANIES ATTENDING*



Statistics taken from 6th RNA-Targeted Drug Discovery & Development Summit


GET INVOLVED





William Hobson-Corbett
Partnerships Director
Tel: +1 617 455 4188
Email: sponsor@hansonwade.com

Ready to Register?

3 Easy Ways to Book

 www.rna-drugdiscovery.com/take-part/register/

 Tel: +1 617 455 4188

 Email: info@hansonwade.com



LEARN and experience how leading companies are unleashing the full potential of screening and structural biology to improve RNA target understanding



DEVELOP your understanding into the current challenges, strategies and solutions, including improving reliability of safety testing to accelerate more RNA targeting small molecules towards the clinic



ENGAGE with your community and peers from leading pharma and biotech companies to build complementary collaborations and partnerships

WELCOME

EXPERT SPEAKERS

AGENDA

PARTNER WITH US

REGISTER YOUR PLACE

Drug Developer Pricing*	Register in Advance to Save \$100	On the Door
Conference + Focus Day OR	\$4,097 (Save \$100)	\$4,197
Conference + Workshop Day	\$4,097 (Save \$100)	\$4,197
Conference Only	\$2,899 (Save \$100)	\$2,999
Academic Pricing**	Register in Advance to Save \$100	On the Door
Conference + Focus Day OR	\$3,497 (Save \$100)	\$3,597
Conference + Workshop Day	\$3,497 (Save \$100)	\$3,597
Conference Only	\$2,499 (Save \$100)	\$2,599
Solution & Service Provider Pricing	Register in Advance to Save \$100	On the Door
Conference + Focus Day OR	\$4,997 (Save \$100)	\$5,097
Conference + Workshop Day	\$4,997 (Save \$100)	\$5,097
Conference Only	\$3,599 (Save \$100)	\$3,699

*To qualify for the drug developer rate your company must have a publicly available drug pipeline and not offer paid for services. Please visit the website for full pricing options or email info@hansonwade.com

**To qualify for academic rate you must be full time academic. Please visit the website for full pricing options or email info@hansonwade.com Do you work for a Not-for-Profit organization? Email us at info@hansonwade.com to inquire about attending



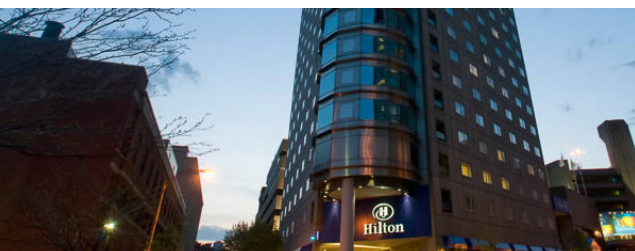
Team Discounts***

- 10% discount – 2 Attendees
- 15% discount – 3 Attendees
- 20% discount – 4 + Attendees

***Please note that discounts are only valid when two or more delegates from one company book and pay at the same time.

Discounts cannot be used in conjunction with any other offer or discount. Only one discount offer may be applied to the current pricing rate.

Contact: info@hansonwade.com



Venue

Hilton Boston Back Bay

40 Dalton Street, Boston, MA 02115, United States

www.hilton.com/en/hotels/bosbh-hilton-boston-back-bay/

TERMS & CONDITIONS

Full payment is due on registration. Cancellation and Substitution Policy: Cancellations must be received in writing. If the cancellation is received more than 14 days before the conference attendees will receive a full credit to a future conference. Cancellations received 14 days or less (including the fourteenth day) prior to the conference will be liable for the full fee. A substitution from the same organization can be made at any time.

Changes to Conference & Agenda: Every reasonable effort will be made to adhere to the event programme as advertised. However, it may be necessary to alter the advertised content, speakers, date, timing, format and/or location of the event. We reserve the right to amend or cancel any event at any time. Hanson Wade is not responsible for any loss or damage or costs incurred as a result of substitution, alteration, postponement or cancellation of an event for any reason and including causes beyond its control including without limitation, acts of God, natural disasters, sabotage, accident, trade or industrial disputes, terrorism or hostilities.

Data Protection: The personal information shown and/or provided by you will be held in a database. It may be used to keep you up to date with developments in your industry. Sometimes your details may be obtained or made available to third parties for marketing purposes. If you do not wish your details to be used for this purpose, please write to: Hanson Wade Ltd, Eastcastle House, 27/28 Eastcastle Street, London, W1W 8DH, United Kingdom