www.rnaediting-summit.com







6th Annual

RNAEditing Summit

Accelerating the Next Wave of RNA Editing Drugs to Patients

Building Novel RNA Editors with Optimized Efficiency, Target Selection, & Extra-Hepatic Delivery to Progress Safe & Efficacious Therapies into the Clinic for Rare & Common Diseases

Expert Speakers Include:



Ram Aiyar Chief Executive



Tehmina Masud Vice President & Head of Systems & Target Biology



TJ Cradick Chief Technology Officer **Therapeutics**



Paul Bolno Chief Executive Officer Vave Life Sciences



Robert Deans Chief Technology Officer



Sriram Sathy Chief Scientific Officer

Proud to Partner With:















Welcome to the 6th RNA Editing Summit



With Wave Life Sciences and Korro Bio advancing drugs into the clinic, new biotech and pharma partnerships developing, and ProQR and AIRNA's recent investment funding, the floodgates have opened for the RNA editing industry, creating new opportunities to expand therapeutic pipelines and forge a path

The 6th RNA Editing Summit returns to Boston as the world's leading industrydedicated forum focused on advancing the discovery and development of RNA modifying therapies, showcasing novel, innovative editing technologies, translational and functional biology and groundbreaking clinical data.

Uniting RNA editing leaders from the likes of Korro Bio, Shape Therapeutics, Deep Genomics, HuidaGene Therapeutics and more, this three day deepdive meeting will focus on:

- Presenting new clinical insights from industry leaders to guide your strategies toward clinical success, helping RNA editors reach patients more quickly
- Showcasing innovative RNA editing technologies, including ADAR, exon splicing, and CRISPR-based editors, to target previously undruggable diseases
- Achieving extra-hepatic delivery with novel carrier formulations for transporting RNA editor payloads of any size, broadening treatment indications
- Exploring functional assays and machine learning for target discovery and design, providing technical insights to accelerate your research

Connect with 60+ experts in Platform Biology, Chemistry and R&D for an unrivalled learning and networking opportunity designed to accelerate preclinical RNA editing candidates towards clinical development, as the next wave of therapies moves ever closer to patients in need.

What's New for 2025?

New Speaking Companies: Deep Genomics, HuidaGene Therapeutics & **Rett Syndrome Research Trust**

CEOs' Fireside Chat with:





Key Benefits of Attending



Maximize modification magnitude by improving editing efficiency, with lessons on designing robust, targeted guide RNA to improve extra-hepatic delivery, increase bioavailability and minimize off-target effects with Korro Bio and ProQR **Therapeutics**



Unleash the potential of RNA editing through a deeper understanding of RNA and ADAR biology, and gain insights into novel editing machinery including multikilobase, edits and cas-based systems, with Amber Bio and **UC Davis**



Achieve the full therapeutic impact of RNA modification by differentiating targets and exploring novel technologies to edit multiple mutations with the scope to expand into common disease, with Deep Genomics, Jorna Therapeutics and Radar Therapeutics

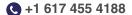


Navigate the path to the clinic by overcoming the limitations of preclinical models, unravelling complex regulatory guidelines, and hearing first-hand success stories from the pioneers of RNA editing, with HuidaGene Therapeutics and Wave Life Sciences



Explore the investment and partnering landscape to attract funding and ensure success in driving investigational RNA editors into clinically and commercially viable therapies, with **Jefferies**. Chardan and Cantor **Fitzgerald**











Your Expert Speakers



RNA Editing Experts



Jacob Borrajo Founder & Chief Executive Officer **Amber Bio**



Sriram Sathy Chief Scientific Officer **AIRNA**



Kris Elverum Chief Executive Officer **AIRNA**



Jeff Wintersinger Senior Computational Biologist **Deep Genomics**



Tehmina Masud Vice President & Head of Systems & Target Biology **Deep Genomics**



Jonathan Gootenberg Assistant Professor **Harvard Medical School**



Omar Abudayyeh Assistant Professor **Harvard Medical School**



TJ Cradick Chief Technology Officer **HuidaGene Therapeutics**



Alan Herbert Founder **InsideOutBio**



Vincent DiGiacomo Director, Therapeutic Innovation Jorna Therapeutics



Maryam Habibian Director, Head of Medicinal Chemistry **Korro Bio**



Ram Aiyar Chief Executive Officer **Korro Bio** IN THE CLINIC



Venkat Krishnamurthy Senior Vice President & Head of Platform, RNA **Editing** Korro Bio

IN THE CLINIC



Gerard Platenburg Chief Scientific Officer **ProQR Therapeutics**



Andrew Fraley Chief Innovation Officer **Radar Therapeutics**



Robert Deans Chief Technology Officer **Rett Syndrome Research** Trust



Yang Gao Assistant Professor **Rice University**



Brian Booth Director, Computational Biology RNAfix **Shape Therapeutics**



Chad Weldy Instructor, Cardiovascular Medicine Stanford University



Peter Nell Chief Executive Officer **Stealth Biotech**



Peter Beal Professor **UC Davis**



Artem Nemudryi Assistant Professor **University of Florida**



Paul Bolno Chief Executive Officer Wave Life Sciences IN THE CLINIC



Anna Dudkina Independent RNA Editing



Steve Seedhouse Biotechnology Equity Research Cantor Fitzgerald

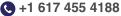


Keay Nakae Director of Research Chardan



Roger Song Senior Equity Research Analyst **Jefferies**







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Pre-Conference Workshop Day Tuesday | July 29, 2025



July, 29-31, 2025 | Boston, MA

Check In & Light Breakfast

8.00

Workshop A

9.00

Developing Robust, Reproducible & Sensitive Analytical & Functional Assays to Accurately Measure Editing Efficiency, Off-Target Activity & Impact of Editing on Disease

Understanding the level of achieved editing efficiency and the functional effect of editing is vital to measure efficacy and understand the true impact of RNA editing therapies. Reliable, reproducible analytical assays are essential in this process, and can indicate off-target editing, leading to the development of safer, more efficacious RNA editing medicines.

This workshop will cover:

- Developing robust, reproducible analytical assays which mimic human biological mechanisms to accurately measure levels of editing efficiency
- Utilising functional assays to understand the effect of editing on protein function and disease to elucidate efficacy
- Analysing the transcriptome and genome to understand off-target effects and inform biomarker identification and future therapeutic development

Workshop Leaders



Anna Dudkina Independent RNA Editing Expert



Alan Herbert Founder InsideOutBio

Lunch & Networking

12.00

Workshop B

1.00

Navigating RNA Editing Regulatory Challenges & Optimizing Translation of RNA Editing Candidates to Accelerate IND Approval, Streamline Entry into the Clinic & Achieve Commercial Success for RNA Editors

Developing clinically successful therapies with strong efficacy and tolerability relies on robust preclinical development and regulatory compliance in order to maximize patient safety and ultimately drive market success. Navigating the regulatory landscape for RNA editing can be challenging, with unforeseen hurdles to overcome on the path to commercialization.

This workshop will address:

- Understanding the attributes necessary for clinical translation of RNA editing candidates
- Navigating the regulatory path and upholding standards during preclinical and clinical development
- Identifying key hurdles on the path to commercialization and strategizing solutions to accelerate success in the clinic and on the market

Workshop Leaders



TJ Cradick
Chief Technology
Officer
HuidaGene
Therapeutics



Robert Deans Chief Technology Officer Rett Sydrome Research Trust

End of Pre-Conference Workshop Day

4.00









Conference Day One Wednesday July 30, 2025





8.30 Check In & Light Breakfast



8.50 Chair's Opening Remarks

Optimizing Editing Efficiency by Designing Durable Guide RNA & Effectively Recruiting Editing Machinery to Target Sites to Increase Magnitude of Modification

9.00 Harnessing Endogenous ADAR for Oligo-Directed RNA Editing



- The OPERA platform (Oligonucleotide Promoted Editing of RNA), which utilizes synthetic oligonucleotides that recruit ADARs to repair disease-causing mutations at the RNA level
- In addition to repairing standard G-to-A mutations, our platform enables the modulation of protein function by changing the amino acid code
- Updates on the OPERA platform and progress towards the clinic

9.30 Advancing ADAR Mediated RNA Editing Therapies: An Optimal Platform for Treatment of Rare & Common Genetic Diseases



- Platform Innovation: RESTORE+ platform leverages proprietary GalNAc delivery and enhanced chemistry to maximize RNA editing potency, durability, and safety
- Targeted Liver Delivery: Engineered for precise ADAR-mediated editing with minimized safety risks, ensuring effective hepatic targeting
- Lead Program in AATD: Focused on addressing the genetic root of Alpha-1 Antitrypsin Deficiency to offer a transformative therapeutic option



10.00 Morning Break & Speed Networking

This is your opportunity to connect and establish meaningful industry relationships with some of the biggest names in RNA editing!

Unleashing the Full Therapeutic Impact of RNA Editing Through Deep Understanding of RNA Structure & ADAR to Ensure Efficacious Editing



11.00 Molecular Basis of ADAR1-Mediated RNA Editing

- Outlining the biochemical profiling of ADAR1's RNA substrate preference
- Comprehending atomic resolution structures of ADAR1
- Understanding the domain requirement for RNA editing

11.30 Structure-Guided Optimization of ADAR-Guiding Oligonucleotides

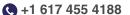


- High resolution structures of ADAR-RNA complexes solved by X-ray crystallography and cryo-EM
- Describing the impact of specific nucleoside analogs at different positions in ADAR guide strands
- Shedding light on the mechanism of the ADAR deamination reaction and informing the design of highly efficient and selective ADAR guide strand for therapeutic editing using chemically modified RNA



12.00 Lunch Break & Networking











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Conference Day One Wednesday July 30, 2025



Exploring Novel Editing Technologies Beyond ADAR to Increase the Scope of RNA Modification & Reach Previously Undruggable Patient Populations



Jacob Borrajo
Founder & Chief
Executive Officer
Amber Bio

1.00 Programmable Multi-Kilobase RNA Editing with Splice Editors

- Amber Bio is building Splice Editors, a novel RNA editing technology
- Exploring the ways Splice Editors can achieve multi-kilobase edits with a single AAV
- Understanding how Splice Editors open the aperture for addressable targets

1.30 Break-and-Repair RNA Editing with CRISPR Ribonucleases



- Sequence-specific RNA cleavage by CRISPR ribonucleases and subsequent RNA repair enables programmable RNA editing
- Break-and-repair RNA editing facilitates engineering of RNA viruses without DNA intermediate
- CRISPR-guided RNA breaks are repaired in human cells, which can be used for programmable RNA excisions to restore gene function



2.00 High Fidelity RNA Editor Development & Studies Supporting the World's First CRISPR/RNA-Targeting Therapy, HG202, for Patients with Neovascular Age-Related Macular Degeneration

- Identification, protein engineering and characterization of high fidelity hfCas12Y
- The opportunity and advantages or RNA editing for Neovascular Age-Related Macular Degeneration (nAMD)
- The molecular composition, mechanism of action, and preclinical animal studies of RNA editor HG202



Omar Abudayyeh Assistant Professor Harvard Medical School

2.30 Programmable RNA Writing with Trans-Splicing

- Utilizing trans-splicing technology to replace large RNA segments, enabling the correction of multiple mutations at once
- Demonstrated correction for transcripts of therapeutically relevant genes, including those implicated in Huntington's Disease and Rett Syndrome
- Protein-free system design allows for efficient AAV delivery, facilitating in vivo RNA correction strategies



Jonathan Gootenberg Assistant Professor Harvard Medical School



3.00 Afternoon Break & Poster Session

Take this opportunity to showcase your latest RNA editing data with your peers and understand the strategies of your fellow experts. Please visit the website for T&Cs of submitting a poster.











Conference Day One Wednesday July 30, 2025



Promoting Specific Targeting & Enhanced RNA Delivery to Extra-Hepatic Targets to Maximize Bioavailability & Reach the Therapeutic Potential of RNA Editing

4.00

Round Table Discussion: Limiting Off-Target Effects by Ensuring Specific Payload Delivery with Highly-Specific Guide RNA to Prioritize Patient Safety





- Utilizing transcriptomic data to assess peripheral sequences and empirically monitor off-target edits
- Designing guide RNA for maximum durability and specificity to limit toxicity and immunogenicity
- · Determining safety thresholds and acceptable levels of off-target editing

4.45 RNAfix Drives Efficient RNA Editing in the Non-Human Primate CNS with IV Delivery



5.15

Brian Booth
Director Computational
Biology RNAfix
Shape Therapeutics

- RNAfix uses ADAR-recruiting guide RNAs to achieve >90% in vivo A-to-I editing
 efficiency in mouse and NHP CNS with exquisite transcriptome-wide specificity
- Shape's novel AAV capsid SHP-DB1 efficiently crosses the NHP blood-brain-barrier following IV administration, delivering RNA editing across the entire brain without invasive cranial injections
- ShapeTX's integrated platform combines Al-optimized RNAfix guide RNAs, proprietary CNS-tropic AAV capsids, and disruptive stable cell line AAV manufacturing to overcome key gene therapy limitations, enabling scalable, cost-effective solutions to address unmet needs in neurological disorders



Chair's Closing Remarks & End of Conference Day One

■■ I look forward to interacting with industry leaders in RNA editing to collaboratively address challenges, identify future directions, and celebrate victories. ▶▶

Chief Executive Officer, Amber Bio

■ The field of RNA editing, especially since it is small, benefits from an exchange of ideas and an honest discussion of the challenges. Any insight at several levels – target classes of interest, the regulatory requirements, the major technology limitations that need to be overcome – are incredibly valuable for a small start-up that can very much integrate the knowledge we take in. ▶ ▶

Director, Therapeutic Innovation, Jorna Therapeutics









Conference Day Two Thursday July 31, 2025



July, 29-31, 2025 | Boston, MA



8.00 Check In & Light Breakfast



8.50 Chair's Opening Remarks

Illuminating the Investment & Partnering Landscape to Guide Future Directions for Research, Growth & Collaboration to Power the Future of RNA Editing

9.00 Investment Panel: Attracting Investment & Securing Preclinical Funding for Early-Stage RNA Editing Therapeutic Candidates

- How is RNA editing viewed by investors and what are the directions for growth?
- · How to successfully gain funding for preclinical candidates by demonstrating the right data
- What are the hurdles and pitfalls to starting an RNA Editing therapeutics company and what are the timelines, collaborations and levels of investment required



Roger Song
Senior Equity Research
Analyst
Jefferies



Steve Seedhouse
Biotechnology Equity
Research
Cantor Fitzgerald



Keay NakaeDirector of Research **Chardan**



Peter Nell
Chief Executive
Officer
Stealth Biotech

10.00 CEOs' Fireside Chat: Lessons from RNA Editing's CEOs on Driving Clinical & Commercial Development to Accelerate RNA Editors to Patients

- Polying into the work of PNA editing's leading
- Delving into the work of RNA editing's leading CEOs to understand their perspectives on the field, ongoing journey to success, and directions for future development
- Learnings to apply to your own RNA editing pipeline and business strategy to accelerate translation and achieve commercial viability



Steve Seedhouse
Biotechnology Equity Research
Cantor Fitzgerald



Ram Aiyar
Chief Executive Officer
Korro Bio



Paul Bolno
Chief Executive Officer
Wave Life Sciences



Kris Elverum
Chief Executive
Officer
AIRNA



11.00 Morning Break & Networking

Advancing RNA Editing Therapeutic Development with Technological Advances & Strategic Collaborations to Evolve the Scope of Editing to Common Disease



Tehmina Masud Vice President & Head of Systems & Target Biology Deep Genomics

12.00 Uncovering Novel Therapeutic Targets for RNA Editing through Human Genetics & AI

- Leveraging human genetic data and AI to identify and prioritize genetic targets for RNA editing
- Investigating the target landscape in common diseases with significant unmet therapeutic needs
- · Identifying opportunities beyond correcting disease-causing mutations

12.30 Round Table Discussion: Employing Machine Learning & Computational Biology to Validate Novel Therapeutic Targets, Design Robust Guide RNA & Predict Functional Effect of Editing, Driving the Development of Safe, Selective Editors

- Utilizing machine learning and computational biology to predict functional effect of editing to assist preclinical development and safeguard patient safety
- Designing high-quality, durable and ultra-specific guide RNA using machine learning tools to improve editing magnitude and safety
- Employing computational tools to apply RNA editing to common disease by targeting multiple mutations and identifying novel therapeutic targets



Tehmina Masud Vice President & Head of Systems & Target Biology Deep Genomics



Jeff Wintersinger Senior Computational Biologist Deep Genomics



Omar Abudayyeh Assistant Professor Harvard Medical School



Jonathan Gootenberg Assistant Professor Harvard Medical School











Conference Day Two Thursday July 31, 2025





1.30 Lunch Break & Networking

Expanding the Scope of RNA Editing by Analyzing Human Genetics Data & Identifying Differentiated Novel Targets to Successfully Treat a Wider Range of Indications

2.30 Panel Discussion: Uncovering Differentiated Targets, Exploring Editing in a Wider Range of Diseases & Identifying Patients Who Could Benefit from RNA Editing

- · Identifying differentiated target sequences using human genomics data and AI
- · Exploring editing ceilings and limitations of various targets
- · Elucidating treatable diseases and identifying patients with the suitable genotype who could benefit from treatment

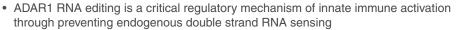


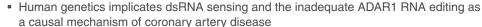
Jacob Borrajo
Founder & Chief Executive Officer
Amber Bio





3.15 Defining ADAR1 RNA Editing as a Causal Mechanism of Coronary Artery Disease





 Through comprehensive studies utilizing human genetics, primary in vitro culture, mouse models of disease, and single cell RNA sequencing, we have defined the mechanism by which ADAR1 RNA editing and dsRNA sensing by MDA5 mediates atherosclerotic disease risk

Chad Weldy Instructor, Cardiovascular

Medicine
Stanford University

Gerard Platenburg Chief Scientific Officer ProQR Therapeutics

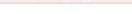
3.45 Pioneering RNA Modification Beyond Rare Diseases by Exploring Novel Editing Technologies & Targeting Multiple Mutations to Remove the Barriers to Treating Common Diseases

- Rethinking editing machinery to edit beyond point mutations and specifically modify multiple target sites simultaneously
- Preclinical data to demonstrate the potential of RNA editing outside of rare disease
- Strategically investigating common disease targets to attract investment and pharma partnerships



Venkat Krishnamurthy Senior Vice President & Head of Platform Korro Bio

4.15 Chair's Closing Remarks & End of 6th RNA Editing Summit











Our 2025 Partners





Industry Partner

Wave Life Sciences is a biotechnology company focused on unlocking the broad potential of RNA medicines to transform human health. Wave's RNA medicines platform combines multiple modalities, chemistry innovation and deep insights in human genetics to deliver scientific breakthroughs that treat both rare and prevalent disorders. Its toolkit of RNA-targeted modalities includes RNA editing, splicing, RNA interference and antisense silencing, providing Wave with unmatched capabilities for sustainably delivering candidates that optimally address disease biology.

www.wavelifesciences.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

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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

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Katie Orchard **Business Development Manager**

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artner With Us



Are You Committed to Supporting the Development Of Efficient RNA Editors With Targeted Delivery?

The 6th RNA Editing Summit is your unique opportunity to position yourself amongst the most innovative players in RNA therapeutics to showcase your solutions and capabilities.

Biopharma is increasingly looking to RNA editing as the next revolutionary modality to alter the outcome of disease, yet challenges in RNA delivery, preclinical development and manufacturing are hindering development.

From advanced analytical testing to oligonucleotide sequencing and synthesis, the pioneers gathering at this summit are looking for solutions to drive the specificity, safety and scalability of their RNA editing candidates - can you help?

Partnership Benefits Include:



Position Yourself as The Leading RNA Solution Provider

Connect with a targeted audience of RNA decisionmakers looking for support to progress the development of this emerging modality



Keep Your Finger on The Pulse in RNA Editing

Gain first-hand insight directly from RNA leaders to better understand the challenges of developing targeted RNA editors so you can efficiently deliver solutions



Build Important Industry Connections With RNA Leaders

Enjoy 8+ hours of dedicated networking time to unite with the biggest names in RNA editing, pioneering start-ups and academic innovators during the poster session and speed-networking breaks



Maximize Your Brand Visibility & Credibility

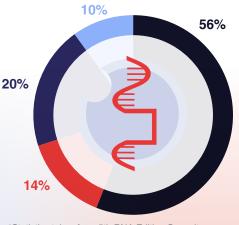
Take advantage of branding opportunities and share your newest data on designing robust guide RNA or delivering to extra-hepatic targets, creating a lasting impression of your capabilities

Seniority of Attendees*

C-level/VP/Director - 68%

Manager/Scientist/ Professor - 32%

Types of Companies Attending*



*Statistics taken from 5th RNA Editing Summit

Drug Developer

Academic & Research Institute

Equipment & Service **Provider**

Other

Get Involved



Katie Orchard

Business Development Manager

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Enhance RNA editing efficiency and reduce off-target modifications with robust guide RNA and optimized extra-hepatic delivery



Expand and differentiate targets for editing with machine learning to treat previously undruggable indications, including common and rare diseases



Explore the investment and partnership landscape to attract funding and collaborations to drive RNA editing therapies to clinical success

Drug Developer Pricing*	Standard Price	On the Door Price
Conference + 2 Workshops	\$4,097 (save \$100)	\$4,197
Conference + 1 Workshop	\$3,498 (save \$100)	\$3,598
Conference Only	\$2,899 (save \$100)	\$2,999
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Conference + 1 Workshop	\$2,998 (save \$100)	\$3,098
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Conference + 1 Workshop	\$4,298 (save \$100)	\$4,398
Conference Only	\$3,599 (save \$100)	\$3,699

^{*}To qualify for the drug developer rate your company must have a public drug pipeline and not offer pay-for services. Please visit the website for full pricing options or email

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- 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

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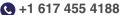
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