

November 19-20, 2019
Boston, USA

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1st RNA Editing Summit 2019

Translating Emerging RNA Editing Approaches for Therapeutic Applications

Expert Speakers Include:



Andrew Fraley
Founder & SVP
Technology
Korro Bio



Joshua Rosenthal
Senior Scientist
Marine Biological Laboratory



Maria Montiel-Gonzalez
Scientist, RNA Editing
Platform
Beam Therapeutics



Thorsten Stafforst
Professor
University of Tuebingen



Scott Ribich
Vice President, Biology
Accent Therapeutics



Jeffrey Ostrove
CEO & Director
Locana



F. Nina Papavasiliou
University Professor &
Division Head, **German Cancer Research Center**, Heidelberg, Germany, & Visiting Professor, **Rockefeller University**



Francois Vigneault
Co-Founder, President & CEO
Shape Therapeutics



David Rodman
Executive VP R&D
ProQR

Welcome to the 1st RNA Editing Summit

RNA editing is reaching an inflection point and is rapidly being discussed, invested in and adopted by the industry. This timely **RNA Editing Summit** is the **first industry led meeting** showcasing the recent developments of RNA editing mechanisms and dedicated to realizing the research and therapeutic opportunity of RNA editing as quickly as possible.

As CRISPR genome editing struggles to overcome key specificity and delivery challenges in its pursuit to realize therapeutic applications, the **1st RNA Editing Summit 2019** accelerates the translation and commercialization of RNA editing for an array of research and therapeutic applications.

As the therapeutic potential of RNA editing explodes, **discover the fundamental mechanisms of RNA editing**, such as recruiting endogenous ADARs with encodable guideRNAs, and learn about the novel approaches being developed to **precisely and efficiently deliver intended RNA edits**. With the temporary nature of RNA editing providing a huge opportunity in many areas beyond genetic diseases, uncover how to recruit endogenous editing enzymes and ultimately deliver safe and effective transcriptome edits.

Join RNA, functional genomic, cell biology and immuno-oncology experts from large pharma, biotech and academia at the **1st RNA Editing Summit 2019** in the pursuit of **translating emerging RNA editing mechanisms into therapeutic reality**.

Why our Speakers are Excited:

■ The field of RNA editing has been exploding the last couple of years, and this inaugural RNA editing summit is going to be a great place for people to hear the science about RNA editors, as well as the different approaches to utilize or inhibit these enzymes for the treatment of human diseases. ■

Scott Ribich, Vice President, Biology, **Accent Therapeutics**

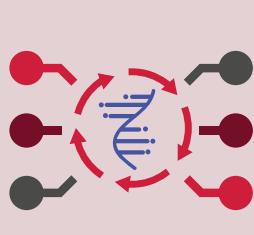
■ RNA editing expands the evolutionary landscape by increasing the diversity of gene products and enhances the therapeutic universe by enabling interventions that go beyond what is possible in a world focused solely on rewriting DNA sequence. This meeting will help expand the collaborations and toolsets for this new era of innovation. ■

Alan Herbert, Founder & Head of Discovery, **InsideOutBio**

The 1st RNA Editing Summit presents the unique opportunity to:



Divulge the current strategies for site-directed RNA editing with a comparison between the different emerging approaches with **Beam Therapeutics** and the **Marine Biological Lab**



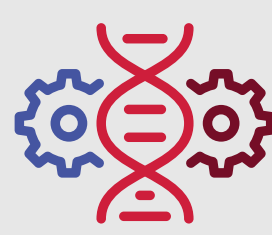
Uncover how RNA editing actually functions and how **Erez Lebanon, Bar-Ilan University** is developing technology and algorithms to reveal A-I RNA Editing in humans and animal models



Explore how RNA editing can be used as a critical immuno-oncology tool with pioneering academics and unravel the different industry approaches to **leverage ADAR in the development of therapeutics** with **Accent Therapeutics, Locana, Shape Therapeutics** and **ProQR**.



Discover how **Thorsten Stafforst** from **Tuebingen University** is engineering editing systems and **harnessing endogenous ADARS with chemically stabilised antisense oligonucleotides** for applications in life sciences and medicine



Dive into **MITs** recent ground-breaking work focused on **RNA editing with CRISPR based systems** and **explore applications of REPAIR and RESCUE** (RNA Editing for Specific C to U Exchange.)

YOUR EXPERT SPEAKERS



Scott Ribich
Vice President,
Biology
**Accent
Therapeutics**



U. Thomas Meier
Professor
**Albert Einstein
College of Medicine**



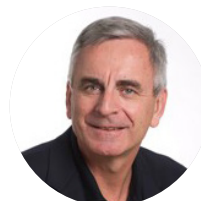
Erez Levanon
Professor
Bar-Ilan University



Maria Montiel-Gonzalez
Scientist, RNA
Editing Platform
Beam Therapeutics



Hyo Min Park
CTO & Co-Founder
GenEdit



Alan Herbert
Founder & Head of
Discovery
InsideOutBio



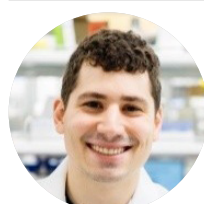
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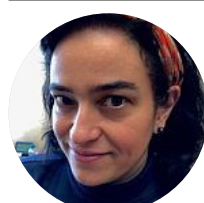
Jonathan Gootenberg
McGovern Fellow,
the McGovern
Institute for Brain
Research
MIT



Omar Abudayyeh
McGovern Fellow,
the McGovern
Institute for Brain
Research
MIT



David Rodman
Executive VP R&D
ProQR



F. Nina Papavasiliou
University Professor &
Division Head, **German
Cancer Research
Center**, Heidelberg,
Germany, & Visiting
Professor, **Rockefeller
University**



Francois Vigneault
Co-Founder,
President & CEO
**Shape
Therapeutics**



Oliver Rausch
Chief Scientific
Officer
**Storm
Therapeutics**



Jin Billy Li
Associate Professor
Stanford University



Eli Eisenberg
Professor
Tel Aviv University



Gene Yeo
Professor
**University of
California, San
Diego**



Prashant Mali
Assistant Professor,
Bioengineering
**University of San
Diego**



Yi-Tao Yu
Principal
Investigator and
Professor
**University of
Rochester Medical
Centre**



Thorsten Stafforst
Professor
**University of
Tuebingen**



Michael Jantsch
Professor
**Medical University
of Vienna**

RNA editing is a rapidly growing field, and I'm excited to see what is presented at the summit

Jonathan Gootenberg, McGovern Fellow, the McGovern Institute for Brain Research, **MIT**

CONFERENCE DAY ONE

TUESDAY NOVEMBER 19 2019

	8.00 Coffee & Registration
Andrew Fraley Founder & SVP Technology Korro Bio	8.30 Chair's Opening Remarks Overview as to why RNA editing is presently reaching an inflection point and the applications that the industry believes RNA editing could tackle
Day Theme: Uncovering How RNA Editing Actually Functions & Exploring the Emerging & Different Approaches for Directed RNA Editing	
Joshua Rosenthal Senior Scientist Marine Biological Laboratory	8.40 Directed RNA Editing, How it has Evolved & General Applications - Limitations of the systems and how to get beyond these - New enzymatic functions and how to discover more - If we can determine C-U editing, we can double the applications - Considerations of longevity of response to tailor for transient and temporary conditions (setting ourselves apart from CRISPR cas 9) - Speculation about the future and where the various systems might be heading
Jonathan Gootenberg McGovern Fellow, the McGovern Institute for Brain Research MIT Omar Abudayyeh McGovern Fellow, the McGovern Institute for Brain Research MIT	9.10 RNA Editing with CRISPR-based Systems - Introducing RNA editing for programmable A to I (G) replacement (REPAIR) - Designing and building a robust RNA editor - Directed evolution of ADAR2 to enable RNA Editing for Specific C to U Exchange (RESCUE) - Applications of REPAIR and RESCUE
Maria Montiel-Gonzalez Scientist, RNA Editing Platform Beam Therapeutics	9.50 Current Strategies for Site-Directed RNA Editing using ADARs - Discuss the different SDRE strategies that have been developed in order to correct G to A genetic mutations and engineer protein function - Difference and comparisons between approaches: - First approach coupling the catalytic domain of ADAR to a guide RNA and a second approach mimicking naturally occurring RNA structures to recruit endogenous ADAR to specific target mRNAs. - Challenges to overcome
	10.20 Morning Refreshments & Speed Networking
Erez Levanon Professor Bar-Ilan University	11.20 Developing Technology & Algorithms to Uncover A-to-I RNA Editing in Humans & Animal Models - RNA editing can be found directly from sequencing data - Algorithms to detect editing events and to distinguish them from mutations and errors are presented - Systematic mapping of the editome across the animal kingdom has revealed that most A-to-I editing sites are located within mobile elements. - Both recoding and non-coding events have implications for genome evolution and, when deregulated, may lead to disease
Thorsten Stafforst Professor University of Tuebingen	11.50 RNA Editing: Engineering Editases, guideRNAs, & ASOs for Application in Life Sciences and Medicine - Engineering editing systems (concept and properties of SNAP-ADARs) - Harnessing endogenous ADARs with chemically stabilized ASOs - Harnessing endogenous ADARs with encodable guideRNAs

Oliver Rausch Chief Scientific Officer Storm Therapeutics	12.20 Targeting RNA Modifying Enzymes for the Treatment of Cancer - Targeting RNA pathways via RNA modifying enzymes - Learnings from Methyltransferases – druggability, efficacy and safety - Overcoming a key challenge in the field: quantitative analysis of RNA modifications using mass spec and other approaches - Perspectives on RNA editing
	12.50 Networking Lunch
Gene Yeo Professor University of California, San Diego	13.50 Identification & Modulation of RNA Binding Protein Binding Sites - Systematic comparisons of A-to-I modulation by CRISPR/Cas formulations - RNA editing to mark RNA binding protein-RNA interaction sites
Prashant Mali Assistant Professor, Bioengineering University of San Diego	14.20 Recruitment of Endogenous and Exogenous ADARs for RNA Editing New Approaches and New Challenges - Genetically encodable RNA Editing platforms - Engineering activity & specificity - In vivo RNA editing
	14.50 Panel Discussion: RNA Off-Target Effects, How Detrimental Are They? - Defining the difference between genomic off targets and RNA off-targets - What level of off targets are permissible? How do we determine this? - How damaging is our guide RNA when it binds to non-specific positions
	15.20 Afternoon Break & Poster Session
U. Thomas Meier Professor Albert Einstein College of Medicine	15.50 Cellular Impact of the RNA Modification Machinery on pre-mRNA Splicing, Ribosome Biogenesis, & Telomere Length Regulation - Subcellular localization of RNPs matters for RNA modification efficiency 2'-O-methylation of snRNAs is important for pre-mRNA splicing - Cajal body localization of the telomerase RNP is important for telomere length regulation - Nopp140 is the underlying principle for Cajal body localization
Yi-Tao Yu Principal Investigator and Professor University of Rochester Medical Centre	16.20 Suppression of Nonsense Mutations in Disease Genes by Targeted RNA Pseudouridylation - Pseudouridylation, which can be catalyzed by RNA-guided mechanism, is the most abundant modification found in RNAs - By changing the guide sequences within the guide RNA, we can re-direct pseudouridylation to new sites in different types of RNA (e.g., the stop codons within mRNA) - Pseudouridylation can profoundly alter the chemical properties of an RNA, thus influencing the contributions of the RNA to cellular process in which it participates - Pseudouridylation at the stop codons can suppress NMD and promote the translation readthrough of nonsense-containing transcripts, thus offering an opportunity to develop a new therapeutic approach to diseases caused by nonsense mutations
Andrew Fraley Founder & SVP Technology Korro Bio	16.50 Chairs Closing Remarks
	17.00 End of Day One at the 1st RNA Editing Summit 2019

CONFERENCE DAY TWO

WEDNESDAY NOVEMBER 20 2019

8.30 Coffee & Registration

9.00 Chair's Opening Remarks

Day Theme: Engineering Applications of RNA Editing & Delving into the Relationship Between ADAR and Immuno-Oncology

Francois Vigneault
Co-Founder,
President & CEO
Shape Therapeutics

9.10 Application of ADAR in Development of Rare Disease Therapeutics

- Why we are using RNA Editing over contemporary genome engineering technologies (CRISPR, TALENs and Zinc Fingers)
- Why is ADAR our focus and how does it solve the problems that CRISPR presented?
- Exploring ShapeTX's technology core platform "RNAfix"

David Rodman
Executive VP R&D
ProQR

9.40 Translating RNA Editing to Our Clinical Development Pipelines

- Introduction to our proprietary platform: Axiomer, a fully owned and in house developed RNA editing platform with very broad applicability
- Harnessing RNA editing in perusing our deep pipeline in ophthalmology
- How this platform is accelerated for precision medicine in the retina with oligonucleotide therapeutics

Jeffrey Ostrove
CEO & Director
Locana

10.10 Harnessing RNA-Binding Proteins to Develop a Portfolio of RNA-Targeting Gene Therapies

- Developing CRISPR-based therapies that target and edit RNA
- Exploring the strategy that could be used to destroy disease-causing RNA transcripts in conditions like myotonic dystrophy and amyotrophic lateral sclerosis
- Looking beyond CRISPR for RNA-binding proteins that could be engineered for making RNA-editing therapies

10.40 Morning Refreshments & Networking Break

Jin Billy Li
Associate Professor
Stanford University

11.20 ADAR1 RNA Editing Evades MDA5-mediated dsRNA Sensing in Innate Immunity

- The embryonic lethality phenotype of Adar1 editing deficient mice is rescued to full life span upon concurrent removal of cytosolic dsRNA sensor MDA5
- ADAR1 p150, not p110 isoform, is responsible for evading the dsRNA sensing by MDA5
- A subset of dsRNA substrates are immunogenic if not sufficiently edited

Michael Jantsch
Professor
University of Vienna

11.50 Local & Global Mechanisms Controlling RNA-Editing

- A to I editing levels vary across the transcriptome-independent of ADAR expression levels
- Editing levels for specific substrates can be controlled by physiological conditions
- Aberrant editing patterns can lead to pathologies
- Controlling cellular physiology may be used to manipulate editing levels

F. Nina Papavasiliou
University Professor
& Division Head,
German Cancer Research Center,
Heidelberg, Germany,
& Visiting Professor,
Rockefeller University

12.20 Realizing RNA Editing as a Critical Immuno-Oncology Tool

- RNA editing and functional relevance for cancer progression
- RNA editing as a diagnostic in cancer progression
- RNA editing in co ntext of immunotherapy

	12.50 Networking Lunch
Eli Eisenberg Professor Tel Aviv University	13.50 The Alu-Editing-Index: Transcriptome-Wide Quantification of ADAR Activity - We present a robust and simple-to-use computational tool to calculate the AEI, a measure quantifying global ADAR activity - The AEI is a diagnostic and prognostic marker in cancer and immunological disorders - The tool can be modified to assess the specificity of directed editing, quantifying off-target editing activity
Scott Ribich Vice President, Biology Accent Therapeutics	14.20 ADAR1 as a Therapeutic Target in Type I Interferon Signaling-High Cancer - A subset of cancer cell lines and tumors displays endogenous elevated Type I IFN signaling - ADAR1 loss induces selective cell killing in these cells through elevated dsRNA signaling by MDA5 (with downstream IFN production) and PKR (and downstream inhibition of translation) - These studies indicate an ADAR1 inhibitor would be a useful therapeutic for cancer with elevated Type I IFN signaling and possibly in combination with existing immunotherapies
Alan Herbert Founder & Head of Discovery InsideOutBio	14.50 ADAR Editing: From 'A' to 'Z' - Introduction: InsideOutBio is a pre-clinical company using complement to increase the immunogenicity of tumors - Many tumors are dependent on ADAR in vitro, relying on this enzyme to downregulate apoptotic responses induced by dysregulated transcription of double-stranded RNA elements. In vivo, inhibition of ADAR amplifies the anti-tumor immune response. Questions: - Which domain of ADAR should we target? - Is it better to target ADAR substrates? - Should we target ADAR's editing-independent functions? - What does ADAR editing tell us about the efficacy of non-ADAR targeted therapeutics?
Hyo Min Park CTO & co-founder GenEdit	15.20 Delivering Next Generation Genetic Therapeutics with Polymer Nanoparticle - GenEdit develops chemically synthesized, non-viral, biopolymer nanoparticles that will enable the efficient in vivo delivery of nucleic acid, protein or RNP cargoes to specific tissues and cell types. - The technology platform is based on synthesizing and screening our nanoparticle libraries for the best in vivo delivery of genetic cargoes to specific tissues such as muscle, brain, and liver. - GenEdit's proprietary nanoparticle technology addresses key delivery issues that have yet to be adequately solved since the inception of gene therapy to treat intractable diseases
	15.50 Panel Discussion: Harnessing RNA Editing As A Basic Tool For The Lab How we can use RNA Editing to drive research and provide an efficient tool to complement those that we have available now?
	16.20 Chairs Closing Remarks
	16.30 End of Day Two and Close of the 1st RNA Editing Summit

■ All of us were taught that transcript RNA should match in sequence the gene that encodes it. Enter editing, which is robust, reproducible, targetable, functionally critical - it's clearly time to update the books! For someone who's been having fun with RNA editing for the last 10+ years, it's exciting to see it hit the limelight, and attract such basic as well as translational interest. I'm very much looking forward to sharing insights with aficionados old and new at what promises to be a fantastic meeting. ■

F. Nina Papavasiliou, University Professor and Division Head, German Cancer Research Center, Heidelberg, Germany, and Visiting Professor, Rockefeller University, New York.

WHY OUR EXPERT SPEAKERS ARE EXCITED:



The field of RNA editing has been exploding the last couple of years, and this inaugural RNA editing summit is going to be a great place for people to hear the science about RNA editors, as well as the different approaches to utilize or inhibit these enzymes for the treatment of human diseases.

Scott Ribich

Vice President, Biology
Accent Therapeutics



Having studied RNA editing for over 10 years, I am fortunate to witness the growth of the RNA editing field from basic research to multiple avenues of therapeutic applications.

Jin Billy Li

Associate Professor
Stanford University



RNA editing expands the evolutionary landscape by increasing the diversity of gene products and enhances the therapeutic universe by enabling interventions that go beyond what is possible in a world focused solely on rewriting DNA sequence. This meeting will help expand the collaborations and toolsets for this new era of innovation.

Alan Herbert

Founder & Head of Discovery
InsideOutBio



RNA editing can dynamically affect cellular and organismic physiology. Understanding the mechanisms involved may provide a new layer to target and manipulate normal and aberrant cellular function.

Michael Jantsch
Professor

Medical University of Vienna



RNA editing is a rapidly growing field, and I'm excited to see what is presented at the summit.

Jonathan Gootenberg

McGovern Fellow, the McGovern Institute for Brain Research
MIT



All of us were taught that transcript RNA should match in sequence the gene that encodes it. Enter editing, which is robust, reproducible, targetable, functionally critical - it's clearly time to update the books! For someone who's been having fun with RNA editing for the last 10+ years, it's exciting to see it hit the limelight, and attract such basic as well as translational interest. I'm very much looking forward to sharing insights with aficionados old and new at what promises to be a fantastic meeting.

F. Nina Papavasiliou

University Professor and Division Head, **German Cancer Research Center**, Heidelberg, Germany, and Visiting Professor, **Rockefeller University**



RNA editing is an exciting new tool in the genome engineering toolbox with distinct advantages over DNA editing and I'm excited to see the latest and greatest developments in the field at the meeting.

Omar Abudayyeh

McGovern Fellow, the McGovern Institute for Brain Research
MIT

PARTNER WITH US

This is the first industry led meeting focused entirely on realizing the clinical and commercial potential of RNA editing. Partner with us to tap into the transcriptomic engineering eruption that is occurring and gain first mover advantage in this space.

In an intimate and highly interactive environment, the **1st RNA Editing Summit** will bring together a truly focused community of RNA, functional genomic, cell biology and immunooncology experts from large pharma, biotech and academia.

Partnering with the **1st RNA Editing Summit** will ensure you capitalize on the market early, cement your position as an industry leader and support the collective mission of translating emerging RNA editing mechanisms into therapeutic reality.

If you have a solution or service that could benefit this thriving and emerging community, let us know. We'd be happy to discuss further the exclusive opportunities on offer to exhibit and speak as a commercial partner

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GET INVOLVED



Harriet Menzel
Partnership Director
Email: Harriet.Menzel@hansonwade.com

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3 EASY WAYS TO BOOK

-  www.rna-editing-summit.com
-  Tel: (+1) 617 455 4188
-  Email: register@hansonwade.com

- 1 Tap into the genetic engineering eruption** that is occurring and gain first mover advantage in this fast emerging space learning from leading biotech's and pioneering academics.
- 2 Discover the fundamental mechanisms of RNA editing** and learn about the novel approaches being developed to precisely and efficiently deliver intended RNA edits.
- 3 Explore how RNA Editing can be used as a critical immuno-oncology tool** and unravel different industry approaches to utilize or inhibit these enzymes for the treatment of human diseases

SECURE YOUR PLACE

Industry Pricing	Register & Pay before Friday, August 2 (Save \$700!)	Standard Pricing
Conference	\$2,199	\$2,899

Academic Pricing	Register & Pay before Friday, August 2 (Save \$400!)	Standard Pricing
Conference	\$1,399	\$1,799

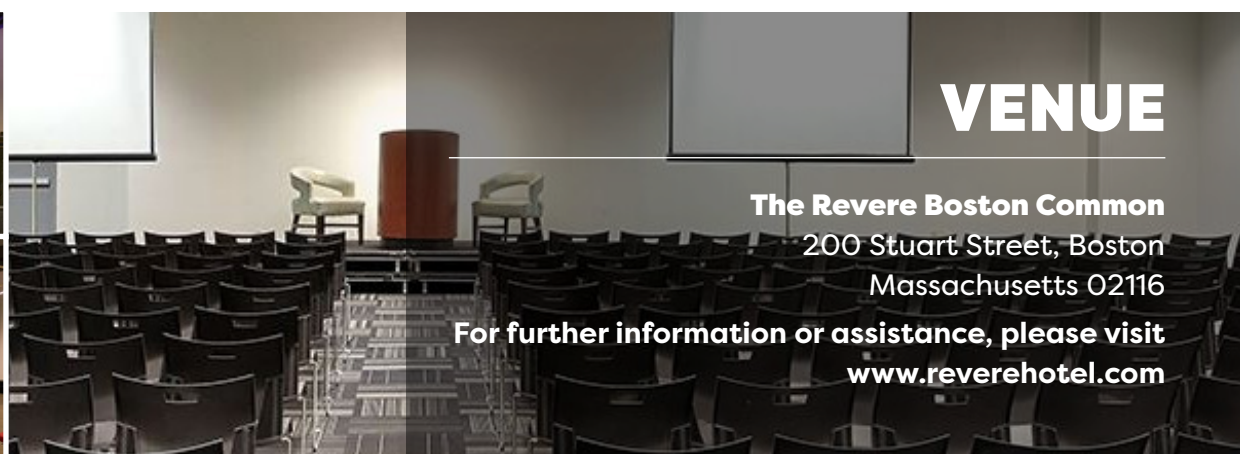
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- **15% discount – 4 delegates**
- **20% discount – 5 or more delegates**

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