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

Research and Markets *
A new paradigm for disease prevention

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

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

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

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

So, if you’re passionate about all things RNA do join us, and more than 150 biotech, pharmaceutical, investment, regulatory and scientific professionals at the RNA Leaders World Congress. You’ll hear the latest advances in RNA chemistry, get clinical updates from the companies developing new therapies and source the services partners who are helping to deliver drugs to patients.
“This fresh approach to tackling genetic diseases has the potential to create a sea change in how we think about treating people with inherited conditions. With safe and effective treatments that really work, genetic diseases that are incurable at present need not be a life sentence in the future. The barriers that remain are not insurmountable. We still have work to do, but the future is bright for RNA therapies.”

Lorna Harries, CSO, Senisca
Meet our partners

Gold Partners

Evonik
LGC
Axolabs
Biocorchestra

Silver Partners

ReCode Therapeutics
Abzu

Bronze Partner

Cend Therapeutics
Remix Therapeutics

Supporting Partners

Charles Consultants
FHM Ford Hutman Media
FreeMind
MediStrava Consulting
Scius Communications
SPE Society of Physician Entrepreneurs
Zyme Communications

www.lsxleaders.com/rna
Meet the speakers

Brett Monia
CEO, Ionis Pharmaceuticals

Douglas Fambrough
CEO, Dicerna Pharmaceuticals

Bob Brown
CSO, Dicerna Pharmaceuticals

Gene Liau
CSO, Stoke Therapeutics

Denis Drygin
CSO, Regulus Therapeutics

Bill Haney
CEO & Chairman, Skyhawk Therapeutics

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

Lorna Harries
Co-Founder and CSO, Senisca

Aimee Jackson
CSO, Atalanta Therapeutics

Samir Ounzain
CEO and Scientific Co-Founder, HAYA Therapeutics

Robert Copeland
CSO, Accent Therapeutics

Laura Sepp-Lorenzino
CSO, Intellia Therapeutics

Dietrich Stephan
Founder and CEO, Neubase Therapeutics

Marcel Blommers
CSO, Saverna Therapeutics

Howard Stern
CSO, Korro Bio

Carsten Rudolph
Co-Founder and CEO, Ethris

www.lsxleaders.com/rna
Meet the speakers

Nathaniel Wang
CEO, Replicate Bioscience

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

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

Shalini Andersson
Head of Oligonucleotide Discovery, AstraZeneca

Tim Luker
VP, S&E – Emerging Technology, Corporate Business Development, Eli Lilly

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

Matthew Disney
Professor, Department of Chemistry, Scripps Research

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

Thorsten Stafforst
Interfaculty Institute of Biochemistry, Eberhard Karls Universität Tübingen

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

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

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

David Slack
CEO, Cend Therapeutics

Branden Ryu
CEO, Biorchestra

Angele Maki
SVP & Head of Business Development, ReCode Therapeutics

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

www.lsxleaders.com/rna
**Agenda**

**Wednesday 16th March 2022**
(All times in CET)

**The bright future of RNA**

09:00 Opening remarks

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

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

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

**Douglas Fambrough**, CEO, Dicerna Pharmaceuticals
**Brett Monia**, CEO, Ionis Pharmaceuticals
**Bill Haney**, CEO & Chairman, Skyhawk Therapeutics
**Alexandra Bause**, Co-Founder, Investment Director & Head of VentureLabs, Apollo Health Ventures

09:55 Keynote panel – Where does RNA sit for Big Pharma?

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

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

**Tim Luker**, VP, Search & Evaluation – Emerging Technology & Innovation, Corporate Business Development, Eli Lilly
**Shalini Andersson**, Chief Scientist, New Therapeutic Modalities and Head of Oligonucleotide Discovery, AstraZeneca
**Sylke Poehling**, Senior Vice President and Global Head, Therapeutic Modalities, Roche
**Sudha Chivukula**, Head of RNA Technology, Research & External Innovation, Sanofi Pasteur

10:40 Morning Break and Oligonetworking Roundtables

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

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

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

Innovation Rapid Fire Session

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

11:30  A new and druggable means to reverse cell senescence through modulation of RNA splicing
Levels of splicing factors change during ageing, compromising our ability to carry out “fine tuning” of gene expression. Senisca is using oligonucleotide-based therapeutic approach to reset splicing factor levels and reverse senescence.

Lorna Harries, Co-Founder and CSO, Senisca

11:45  Enabling mRNA therapies for pulmonary disease indications
mRNA therapies can help to replace or augment missing or non-functional proteins or to introduce new proteins to modulate the course of a disease. Ethris are developing a pipeline of immune-modulation and mRNA-based protein replacement therapies.

Carsten Rudolph, Co-Founder and CEO, Ethris

12:00  Targeting long non-coding RNAs for tissue-specific treatment of fibrotic diseases
Specific long non-coding RNAs (lncRNAs) are important regulators of the resting fibroblast to myofibroblast conversion. HAYA is utilising modified ASOs to target and inhibit proprietary lncRNAs to prevent and reverse fibrosis.

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

12:15  Lunch

Delivery and distribution

14:00  New advances in RNA-targeted therapeutics
A leader in antisense technology, Ionis have experience in delivering therapies by nearly all routes of tissue-specific administration. This session covers learnings and new advances in RNA-targeted therapeutics.

- Advances in drug delivery methods
- Addressing intra-cellular trafficking
- What is next? Exploring new chemistries and technology advancements

Brett Monia, CEO, Ionis Pharmaceuticals

14:20  Advancing extra-hepatic delivery of RNAi
With an advanced pipeline of RNAi therapies, Dicerna are making strides in the delivery of tissues beyond the liver. This session covers new leanings and gains in this field.

- Update on clinical pipeline including IND process
- Advances in hepatic and extra-hepatic delivery methods
- Presentation of extra-hepatic delivery data

Bob Brown, CSO, Dicerna Pharmaceuticals
Agenda

14:40 RNA therapeutics enable expansion of disease target space

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

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

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

15:00 Harnessing tumor-targeting iRGD peptides to effectively target and deliver RNA-based therapeutics into solid tumors

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

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

David Slack, CEO, Cend Therapeutics

15:20 Talk topic to be advised

Branden Ryu, CEO, Biorchestra

15:40 Afternoon Break and Services Clinics

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

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

Delivery and durability

16:10 Putting patients at the heart of an saRNA drug delivery strategy

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

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

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

16:30 Talk topic to be advised

Speaker to be advised, Evonik
Agenda

16:50  
Talk topic to be advised

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

17:10  
Advances in RNA-targeted delivery approaches to access previously undruggable targets

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

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

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

17:30  
Improving responses of mRNA using self-replicating RNAs

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

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

Nathaniel Wang, CEO, Replicate Bioscience

17:50  
Famous Pairs Party

RNA Leaders is all about partnerships! Celebrate famous partnerships throughout history at the Famous Pairs Party and enjoy a drink with prospective business partners at the same time. Most importantly, can you find your own famous counterpart at the party?
Thursday 17th March 2022
(All times in CET)

**RNAi advancements**

**09:00**  
Lessons learned from preclinical and clinical development of microRNA therapies  
*Dysregulated microRNA expression is a key factor in many complex multi-factorial diseases. This session explores Regulus’s current pipeline of therapies in development including lessons during clinical progression.*  
- Update on preclinical and clinical pipeline  
- Investigating new disease areas including delivery methods  
- Regulatory challenges and key lessons for development of RNA therapies  
*Denis Drygin, CSO, Regulus Therapeutics*

**09:30**  
A new modality to improve distribution, durability and potency of RNAi  
*The need to treat neurodegenerative diseases is urgent and currently unsolved. This session explores a breakthrough therapeutic approach using RNAi to halt neurodegenerative conditions at their source.*  
- Understanding the modality including impact on distribution, durability and potency  
- Update on pre-clinical development data  
- Tackling delivery and distribution to the deep brain structure  
*Aimee Jackson, CSO, Atalanta Therapeutics*

**10:00**  
Harnessing new chemistries and molecular design to silence disease-associated genes  
*By harnessing the body’s natural RNA interference mechanism, it is possible to create precision medicines that silence disease-associated genes. This session looks at new chemistries and molecular design to tackle a wide range of diseases.*  
- Putting patients at the heart of new discoveries  
- Update on R&D pipeline and presentation of data  
*Mariah Wikstrom Lindholm, SVP and Head of Molecular Design, Silence Therapeutics*

**10:30**  
Morning Break and Oligonetworking Roundtables  
*Are you new to the field? Or would you like to meet some new players? Join the Oligonetworking Roundtables to meet with other RNA leaders over a coffee.*  
*A relaxed structured networking session for those who want to build new business relationships in this growing field.*

**Antisense approaches**

**11:20**  
Talk topic to be advised  
*Speaker to be advised, Axolabs*
Agenda

11:40  Upregulation of protein expression via ASO therapy

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

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

**Gene Liau, CSO, Stoke Therapeutics**

12:10  Drugging the genome to address base causality with a novel synthetic genetic medicine

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

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

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

12:30  Lunch

Gene therapy and editing approaches

14:00  Delivering on the promise of genetic medicine by co-opting the body’s natural RNA editing system

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

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

**Howard Stern, CSO, Korro Bio**

14:25  CRISPR as therapy: a non-viral approach to gene editing

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

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

**Laura Sepp-Lorenzino, CSO, Intellia Therapeutics**
Harnessing ADAR-mediated RNA base editing for therapy

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

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

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

Targeting RNA with small molecules

15:15 Talk topic to be advised

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

15:40 Afternoon Break and Services Clinics

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

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16:10 Drugging the undruggable: correcting the underlying genetics of disease at the RNA level

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

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

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

16:35 Rational approach to designing selective therapeutics targeting RNA

Talk abstract to be advised

Matthew Disney, Professor, Department of Chemistry, Scripps Research

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

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

- Synergies and complementarity to the rapidly evolving RNA therapeutic approaches
- Assays and screening assays
- A look at recent and upcoming work including collaboration between Big Pharma and academia

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

17:25  Early drug discovery of novel RNA drug targets

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

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

Marcel Blommers, CSO, Saverna Therapeutics

17:50  Close of congress
A multibillion-pound market

The Opportunity

- **500+** companies actively developing RNA therapeutics
- **4000+** live clinical trials for RNA therapeutics & vaccines
- **1500+** investors actively looking to fund RNA technology
- **96** successful fund raises by mid 2021 (vs 101 total in 2020)

The Market

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<tr>
<th>RNAi</th>
<th>Antisense technologies</th>
<th>Targeting with small molecule</th>
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<td>Dicerna</td>
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“Global mRNA vaccines and therapeutics market to reach...”
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<th><strong>2 major mRNA</strong> vaccine approvals in 2020</th>
<th><strong>3 RNAi</strong> therapeutic approvals in the past 4 years</th>
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<td>More than $1.65bn raised by RNA biotech mid 2021</td>
<td><strong>Millions</strong> of lives saved by mRNA vaccines to date</td>
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**RNA delivery**

- bicycle therapeutics
- optimeos life sciences inc.
- ARCTURUS therapeutics
- Spartina biotechnologies
- BIODERCHERSTRA

**mRNA**

- moderna
- BIONTECH
- Cend Therapeutics
- Translate BIO
- Pantherna Therapeutics GmbH

**Discovery tools**

- AVIDITY BIOSCIENCES
- VesigenTx
- PRECISION NANO SYSTEMS
- CUREVAC
- genuity science

*Global mRNA vaccines and therapeutics market to reach $15.49bn by 2026, spurred by increasing investments in biotechnology*
“It was a moment like no other — once in a lifetime — when you’ve been involved in 16 years’ worth of effort to bring an important innovation forward for patients, and finally have it approved”

John Maraganore, CEO, Alnylam Therapeutics
Why Partner with RNA Leaders?

Proven track record

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

Commitment to good science

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

Helping sponsors to present themselves well

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

A valuable investor network

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

Virtual and physical experience

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

First movers in an emerging field

- Bringing people face-to-face again
- Showcasing developments across all RNA medicines
A continued working partnership

Early sponsors confirmed

Key sponsors announced

Programme launch

Your logo on event materials

Webinar

Program in development

Your experts give input to the program

Email campaign to LSX database

Promotion via external media partners

1200+ C-suite database

13k Social media followers
Your company hosts a webinar

Webinar

Your speaker presents on stage
Your logo highly visible onsite
Your sales team exhibits
Your clients attend as guests

RNA Leaders

Post-event promotion

Promotion via RNA Insider newsletter

Speakers promoted on email and social

Your company listed as a 2022 sponsor

1000+ newsletter subscribers

200+ average webinar viewers
Why sponsor?

Thought Leadership

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

Education

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

Branding

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

Business Development

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

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

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

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

To take part, contact joe@lsxleaders.com
# Packages & pricing

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<th>Industry Tickets</th>
<th>Before 19-Nov</th>
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<td><strong>Start-Up Biotech (&lt;3yrs) Ticket</strong></td>
<td>€1,140</td>
<td>€1,290</td>
<td>€1,440</td>
<td>€1,590</td>
<td>€1,740</td>
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<td>• Two full conference days (16-17 March)</td>
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<tr>
<td>• Access to networking app</td>
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<td>• Lunch &amp; refreshments</td>
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<td>• Access to networking function</td>
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<tr>
<td><strong>Academic / Research Institute / NFP Ticket</strong></td>
<td>€840</td>
<td>€890</td>
<td>€940</td>
<td>€990</td>
<td>€1,040</td>
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<tr>
<td>• Two full conference days (16-17 March)</td>
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</table>

## Discounts on tickets for groups

<table>
<thead>
<tr>
<th>Discount</th>
<th>Percentage</th>
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<tbody>
<tr>
<td>3+</td>
<td>save an extra 20%</td>
</tr>
<tr>
<td>5+</td>
<td>save an extra 30%</td>
</tr>
</tbody>
</table>

Contact joe@lsxleaders.com for the discount code!

Book your tickets at [www.lsxleaders.com/rna](http://www.lsxleaders.com/rna)
Contact the team

For questions about sponsoring
Joe Knight
Business Development Manager
joe@lsxleaders.com

For questions about marketing
Paul Gilbertson
Chief Marketing Officer
paul@lsxleaders.com

For questions about anything else
Angela Tyrrell
Senior Vice President
angela@lsxleaders.com

Or visit us at
www.lsxleaders.com/rna