2nd RNA Therapeutics Summit '17

BARCELONA, SPAIN | OCTOBER 19 - 20, 2017

Key Practical Learning Points of the Summit:

- Developments of mRNA-based therapeutics and delivery systems
- Challenges affecting targeted delivery and overcoming biological barriers
- mRNA-based therapeutic vaccines and drugs
- Current challenges in antisense technologies
- Understanding the implications and limitations of CRISPR
- Strategies in battling cancer and current developments
- A look into therapeutic applications for a bright future ahead
- Modification of DNA and RNA, inhibiting and exhibiting of the genome

Key Speakers:

Dr. Mikko Hölttä, SE
Senior Research Scientist
AstraZeneca

Katja Wosikowski, GE
Director Preclinical Operations
Isarna Therapeutics

Dr. Nicole Meisner-Kober, CH
Senior Investigator, RNA Biology
Developmental & Molecular Pathways
Novartis Institutes for Biomedical Research

Roel Schaapveld, NE
Chief Executive Officer
InteRNA

Dr.Roxana Redis, NE
Senior Scientist
ProQR Therapeutics NV

Dr. Ekkehard Leberer, GE
Senior Director of R&D
Alliance Management
Sanofi

Dr. Martin Piest, NE
Senior Formulation Scientist
Patheon

Dr. Amotz Shemi, IS
CEO
Silenseed

Dr. Melvin Evers, NE
Senior Scientist
uniQure

Dr. Guillaume Pavlovic, FR
Department Head - Genetic Engineering
& Model Validation Department
Phenomin - ICS
(former Institut Clinique de la Souris)

MORE TO BE ANNOUNCED

VONLANTHEN CONFERENCES

BARCELONA, Spain
October 19-20, 2017

2nd RNA Therapeutics Summit '17

*We at Vonlanthen are pleased to announce our **'2nd Annual RNA Therapeutics Summit'** this coming October 19th-20th, scheduled on in Barcelona, Spain.*

***We will be exploring** in-depth the new developments being addressed in RNA therapies. With new interest from developers and investors, this field of medicine is taking on an ever-growing interest. We will see a whole new outlook and methodology from how we treat medicine with the entire abolition of compound drugs as the body becomes more and more closer to being its own saviour.*

***We will be taking** a comprehensive look into the different strategies of how we can overcome challenges in delivery? What does the future hold for CRISPR? Is the way we treat cancer and viruses today soon to be a thing of the past?*

***All of this and more** shall be explored among a panel of 14 expert speakers who are pioneering the evolution of RNA therapies. We look forward to seeing you there!*

Who Should Attend:

VPs, CEOs, Directors, CTOs, CSOs, Senior Scientists, Senior Investigators, Senior Biologists, Principal Scientists, Professors, Lab Heads, Heads and Team Leaders, Senior Management and others including:

- Gene, Antisense and RNA Therapy
- Nanomedicine
- Clinical Research
- Molecular Biology
- Human Biology
- Oncology
- Cell Biology
- Assay Development
- Silencing
- Sequencing
- Cancer Immunotherapeutics & Tumour Immunology
- Genomics
- Bioanalysis
- Toxicology
- Drug Delivery
- Drug Development
- Drug Safety
- Translational Medicine
- Tools and Applications Development
- Computational Biology and Bioinformatics
- Molecular Biology
- Vaccines and Diagnostics
- Biochemistry
- Biotechnology
- R&D
- Precision Medicine

About Us

Vonlanthen Group of Companies is made for innovative and senior business leaders focused on confronting challenges and seizing opportunities. We conduct extensive research and connect deal-makers and risk-takers across Europe and emerging markets to help propel companies to the next level. Our conferences, events and training schemes are designed for senior decision-makers working at the top of their industries with cutting-edge strategies, products, processes and technologies.

Vonlanthen Group of Companies is the natural home for companies always on the lookout for opportunities, always searching for the next deal, and always with an eye on the competition.

VONLANTHEN CONFERENCES

BARCELONA, Spain
October 19-20, 2017 ~ DAY ONE

2nd RNA Therapeutics '17 Summit

1 2

08:30 REGISTRATION AND WELCOME COFFEE
09:00 OPENING ADDRESS FROM THE CHAIRMAN

CURRENT ISSUES IN RNA THERAPEUTICS' DELIVERY AND DEVELOPMENT

09:10

CASE STUDY

MicroRNA therapeutics: Targeting the genomic dark matter in human disease

Dr. Ekkehard Leberer
Senior Director of R&D
Alliance Management
Sanofi

- MicroRNAs are short non-coding RNAs that regulate biochemical pathways and networks of pathways by the mechanism of RNA interference (RNAi)
- MicroRNA-21 has been implicated in multiple organs as a microRNA associated with fibrosis and cancer
- The presentation will summarise the opportunities and challenges of developing oligonucleotide therapeutics including microRNA-based drugs and will illustrate the successful generation of an anti-fibrotic microRNA-based therapeutic approach by targeting microRNA-21 with an antisense oligonucleotide (anti-miR-21)

09:50 **SPEED NETWORKING:** An innovative approach to maximize networking capabilities through two minute periods, where delegates can meet their peers and exchange business cards before rotating to the next company representative.

10:30

CASE STUDY

Boronic acid functionalised polymers for the delivery of therapeutic oligonucleotides

Dr. Martin Piest
Senior Formulation Scientist
Patheon

- Tailoring polymers for improved intracellular delivery of oligonucleotides
- Bioreducible polymers with peptide mimicking properties
- Role of boronic acids in nanoparticle formation and transfection
- New possibilities for co-administration of oligonucleotides and small molecules

11:10 MORNING COFFEE AND NETWORKING BREAK

11:40

CASE STUDY

Small interfering RNAs, such as naturally occurring microRNAs and the role they play in almost all aspects of biology

Roel Schaapveld
Chief Executive Officer
InteRNA

- Promising new drug candidates with a great potential to target currently undruggable genes
- The link between microRNA dysregulation and the development of various types of cancer
- Multiple mRNA targets which safely and selectively improve long-term control of advanced tumours and reduce relapse rates
- Agnostic lentiviral-based functional screening in tumour cell-based assays, in order to identify the best microRNA candidates
- Tackling the major elements of the 'hallmarks of cancer'

12:20

CASE STUDY

A highway to the sites of RNA silencing: Cell entry routes of exosomes as a novel paradigm for therapeutic RNA delivery

Dr. Nicole Meisner-Kober
Senior Investigator, RNA Biology
Developmental & Molecular Pathways
Novartis Institutes for Biomedical Research

TO BE ANNOUNCED

13:00 BUSINESS LUNCH

12:20 / 13:00 CASE STUDY BUSINESS LUNCH

UNCOVERING THE POTENTIAL OF RNA CANCER THERAPIES

14:00 CASE STUDY

RNAi in the clinic - phase 2 in pancreatic cancer with/without immune-oncology

Dr. Amotz Shemi
CEO
Silenseed

Silenseed

- siG12D-LODER enables direct targeting of the 'undruggable' oncogene KRAS
- Showing high safety profile and early efficacy evidence in pancreatic cancer
- Phase 2 controlled study siG12D-LODER + chemotherapy to assess efficacy
- Phase 2a of siG12D-LODER + immune oncology

14:40 CASE STUDY

RNA nanomedicine immunotherapy in the fight against cancer and tumours

SPEAKER TO BE ANNOUNCED

TO BE ANNOUNCED

15:20 AFTERNOON COFFEE AND NETWORKING BREAK

THERAPEUTIC APPLICATIONS FOR DISEASE

15:50 CASE STUDY

Genetic manipulation of exosomes as a new therapeutic approach in treating cancer

SPEAKER TO BE ANNOUNCED

TO BE ANNOUNCED

16:30 INTERACTIVE PANEL DISCUSSION

How will delivery systems advance into the next decade?

DETAILS TO BE ANNOUNCED

17:00 CHAIRMAN'S CLOSING REMARKS AND END OF DAY ONE

18:00 BUSINESS DINNER

What We Do

Vonlanthen Group of Companies is the premier forum for deal-makers and business leaders. We help industry experts and investors find the next opportunity, strike the next deal and enter growing markets by:

- Hosting summits, conferences and workshops for senior decision makers, with a focus on sharing practical advice and experience to source opportunities and confront challenges
- Putting top executives together to share insights on the outlook for their industry in our cutting edge leadership forums
- Helping businesses - large and small - fund investment and growth by arranging capital-raising meetings
- Conducting bespoke executive training courses to ensure management teams are operating at the highest possible level

Everybody who attends a Vonlanthen Group event has been pre-screened to ensure the highest quality of delegates and to kick-start the deal-making process.

09:00 REGISTRATION AND WELCOME COFFEE
09:40 OPENING ADDRESS FROM THE CHAIRMAN

THERAPEUTIC APPLICATIONS FOR DISEASE

09:50

CASE STUDY

Establishing proof-of-concept in hemophilia and Huntington's disease

Dr. Melvin Evers
Senior Scientist
uniQure

- uniQure is advancing a focused pipeline of innovative gene therapies and established clinical proof-of-concept in hemophilia B and preclinical proof-of-concept in Huntington's disease
- For Huntington's disease, the therapeutic goal of the gene therapy candidate AMT-130 is to inhibit the production of the mutant protein by a one-time treatment
- Using adeno-associated viral vector to deliver microRNAs directly to the brain represents a highly innovative approach to treating Huntington's disease
- Widespread adeno-associated viral vector distribution with long-term transgene expression and tolerability upon intracranial injection in large animal brain was observed
- Preclinical proof-of-concept studies showed strong target engagement and prevention of neuronal dysfunction in rodent models of Huntington's disease

10:30

CASE STUDY

Axiomer® technology: Therapeutic oligonucleotides for directing site-specific A-to-I editing by endogenous ADAR enzymes

Dr.Roxana Redis
Senior Scientist
ProQR Therapeutics NV

- Recruitment of endogenous RNA editing enzymes by oligonucleotides represents a significant therapeutic opportunity for a new type of drug that can treat genetic disorders by reversing the underlying mutations
- Deamination of adenosines into inosines (A-to-I editing) is catalysed by the ADAR enzymes (adenosine deaminases acting on RNA), and takes place on different substrates, including (pre-)mRNAs, miRNAs and lncRNAs, and in a range of disease-relevant tissues
- To employ ADARs for therapeutic applications, we have developed the Axiomer® platform, which takes advantage of editing oligonucleotides (EONs) containing specific patterns of chemical modifications
- The modifications provide for stability and cellular uptake, and enable the EONs to recruit the endogenous ADARs and direct them to specifically edit one selected adenosine, while suppressing the editing of other off-target adenosines
- We provide proof of concept for the Axiomer® technology in a mouse model of the Hurler syndrome, a lysosomal storage disorder caused by inactivation of the alpha-L-iduronidase enzyme; as well as discuss future applications of the technology

11:10 MORNING COFFEE AND NETWORKING BREAK

11:40

CASE STUDY

Novel approaches for the therapeutic targeting of heart disease through incRNA manipulation

SPEAKER TO BE ANNOUNCED

TO BE ANNOUNCED

12:20

CASE STUDY

Preclincial evaluation of ISTH0036, a LNA-modified antisense oligonucleotide (ASO) specifically targeting the sequence of TGF-β2 mRNA in ophthalmic diseases

Katja Wosikowski
Director Preclinical Operations
Isarna Therapeutics

- The role of TGF-β2 in ophthalmology
- Long lasting tissue biodistribution of ISTH0036 and TGF-β2 mRNA downregulation in posterior eye tissues
- Efficacy in murine CNV model

13:00 BUSINESS LUNCH

12:20 / 13:00 CASE STUDY
BUSINESS LUNCH

INNOVATIVE APPROACHES AND METHODS IN BIOANALYSIS AND RESEARCH

14:00 CASE STUDY

Bioanalytical strategies for measuring RNA therapeutics in complex systems

Dr. Mikko Hölttä
Senior Research Scientist
AstraZeneca

- Workflows & key learnings for measuring modified mRNA and RNA therapeutics
- Techniques for the measurement of microRNA as safety biomarkers
- Approaches taken by AstraZeneca for the measurements of conjugated oligonucleotides
- Problems and pitfalls sharing

14:40 CASE STUDY

The efficiency and simplicity of CRISPR/Cas9 technology to make precise changes to the genome of living cells has led to a new revolution in genetics

Dr. Guillaume Pavlovic
Department Head - Genetic Engineering & Model Validation Department
Phenomin - ICS
(former Institut Clinique de la Souris)

- CRISPR/Cas9 genome editing opens new possibilities for modelling human disease in rodents like easy generation of structural variant models and large humanisation of large locus in mouse
- Scientists are concerned by CRISPR/Cas9 mediated off-targets but other CRISPR drawbacks are poorly evaluated
- Especially, careful characterisation of mouse and rat generated by CRISPR/Cas9 show unexpected mutations like concatemers, ssODN random integration or chromosomal rearrangements
- Finally, we will discuss how rodents can be used to evaluate safety of CRISPR/Cas9 therapeutic approaches especially for unexpected genome modifications

15:20 AFTERNOON COFFEE AND NETWORKING BREAK

15:50 SPONSORSHIP OPPORTUNITIES

SPONSOR SLOT — DETAILS TO BE ANNOUNCED

16:30 INTERACTIVE PANEL DISCUSSION

What are the social implications of RNA?

DETAILS TO BE ANNOUNCED

17:00 CHAIRMAN'S CLOSING REMARKS AND END OF SUMMIT

Sponsorship

Vonlanthen Group of Companies has extensive contacts with key decision makers at the world's biggest companies. Our events and conferences bring industry leaders, deal makers, financiers and investors under one roof, providing you with unique sponsorship and branding opportunities that can deliver an immediate impact and put your message in front of a targeted, specialist audience.

To get your company, product or service in front of a dedicated and engaged group of business leaders, contact us to discuss:

- Conference exhibition opportunities to target new customers, enhance your reputation and strengthen relationships with existing clients
- Event and conference sponsorship with branded materials and speaking opportunities
- Webinars, podcasts, virtual conferences and short videos to generate leads, produce branded content and share your expertise
- Using customer insights and feedback from social media to enhance product and service offerings

All our sponsorship opportunities are bespoke and developed to enhance value for both our partners and Vonlanthen's network of business leaders.

Dr. Mikko Hölttä, SE

Senior Research Scientist
AstraZeneca

Dr. Mikko Hölttä joined AstraZeneca Gothenburg in March 2015 as senior scientist in the nucleotide bioanalysis group, working with pre-clinical PK/TK bioanalysis for new modality projects including oligonucleotides, modified RNA, CRISPR-CAS9, anti-miR, and proteins/peptides. Since March 2017, he has been working in the translational biomarkers & bioanalysis team in AZ Gothenburg providing analytical support for new modality projects and with development and validation of biomarkers for safety studies.
He received his Ph.D. in medicine in 2014, working with clinical biomarkers discovery for Alzheimer's disease and other neurogenerative diseases from Sahlgrenska Academy, University of Gothenburg, and then continued to work as a research scientist in neuroscience & physiology. He has a master's degree in pharmaceutical biosciences from Sahlgrenska Academy in 2008.

Dr. Martin Piest, NE

Senior Formulation Scientist
Patheon

Dr. Ir. Martin Piest holds a Ph.D. in polymer chemistry. He worked on the development of biodegradable polymers for gene therapy and injectable sustained release systems of therapeutic proteins. Currently, he works as senior formulation scientist for Patheon Softgels B.V. on the development of lipidic SEDDS and SMEDDS formulations.

Roel Schaapveld, NE

Chief Executive Officer
InteRNA

Roel served as chief operating officer when InteRNA became operational until he was appointed chief executive officer in May 2009. Prior to InteRNA, Roel was senior manager in the corporate finance biotech team at the Dutch merchant bank Kempen & Co involved in private funding, M&A and IPO's at Euronext of life sciences companies. Previously, he held several management positions at Kreatech Diagnostics (sold to Leica Biosystems), among which include VP corporate and business development. Roel holds M.Sc. and Ph.D. degrees from Radboud University Nijmegen (NL). During his academic career, he worked at the Roche Institute for Molecular Biology (Nutley, NJ), at the Dana-Farber Cancer Institute (Boston, MA) and at the Netherlands Cancer Institute in Amsterdam. Roel received his MBA from Nyenrode Business School (NL).

Dr. Nicole Meisner-Kober, CH

Senior Investigator, RNA Biology
Developmental & Molecular Pathways
Novartis Institutes for Biomedical Research

Nicole Meisner-Kober is a senior investigator at the Novartis Institutes of Biomedical Research. Her research exploits chemical biology to resolve basic mechanistic questions related to RNA therapeutics, RNA silencing biology, and pathological RNA dysregulation. Currently, her group focuses on discovery of new medicines targeting RNA pathobiology in neuro¬muscular diseases. She is also interested in the biology of extracellular vesicles and mechanisms for transfer of RNA cargo between different cells and tissues.

Dr. Ekkehard Leberer, GE

Senior Director of R&D
Alliance Management
Sanofi

Dr. Leberer received his Ph.D. in biology at the University of Konstanz, Germany (1986). He conducted post-doctoral training in molecular biology at the Banting and Best Institute of the University of Toronto, Canada, and then became a professor of biochemistry at the University of Konstanz, Germany (1992). He is currently responsible for R&D alliance management at Sanofi and is the scientific managing director of the innovative medicine initiative COMPACT consortium on the delivery of biopharmaceuticals across biological barriers and cellular membranes (www.compact-research.org).
Since joining Hoechst Marion Roussel in 1998, Dr Leberer carried out various managing roles in this company, Sanofi's predecessor companies and Sanofi itself, including responsibilities in functional genomics, biological sciences and external innovation for oligonucleotide-based therapeutics. He has also served as head of biotechnology Germany and is a member of the Scientific Review Committee of Aventis Pharma Germany.
Prior to joining the pharmaceutical industry, Dr Leberer served as senior research officer in genetics and genomics at the Biotechnology Research Institute, National Research Council of Canada, Montreal. His research has focused on the molecular mechanisms of signal transduction and the role of signalling molecules in human diseases. He is the principal discoverer of the p21 activated protein kinase (PAK) family of cell signalling proteins and of novel virulence-inducing genes in pathogenic fungi. He is the co-author of more than 60 publications in prestigious peer-reviewed journals including, 'Nature and Science.'

Katja Wosikowski, GE

Director Preclinical Operations
Isarna Therapeutics

Katja Wosikowski currently works for Isarna Therapeutics as director preclinical operations, where she is responsible for advancing Isarna´s 2nd generation antisense molecules into the clinic. Besides being a licensed pharmacist, she holds a doctorate in cellular biology from the University of Basel and did a 5-year postdoctoral research stay at the National Cancer Institute, Bethesda, USA.
During her 20+ years of experience in drug discovery and development, she has been in positions with increasing responsibilities in biopharmaceutical companies, managing and leading preclinical development projects of innovative compounds including small molecule inhibitors, antibodies and antisense molecules, as well as herbal drugs. During this time, she gathered experience in different therapeutic fields such as oncology, ophthalmology, and respiratory diseases.

Dr. Melvin Evers, NE

Senior Scientist
uniQure

As senior scientist in research and development of uniQure, Melvin is responsible for the research of the Huntington's disease project. Dr. Evers is also involved in the preclinical pipeline for discovery and developing of new targets for gene therapy using the modular platform to rapidly bring new disease modifying therapies to patients with severe genetic diseases.
He holds a Ph.D. from Leiden University Medical Center from the human genetics department and a M.Sc. from the University Medical Center Utrecht. During his Ph.D. program, he worked with Prosensa on the preclinical development of an antisense oligonucleotide-based therapy for polyglutamine disorders.

Dr.Roxana Redis, NE
Senior Scientist
ProQR Therapeutics NV

Dr. Roxana Redis earned her Ph.D. from the University of Medicine and Pharmacy, Cluj-Napoca, RO in 2013. Her work focused on deciphering the involvement of non-codingRNAs in cancer initiation and progression and it was performed in collaboration with the Erasmus MC in Rotterdam, NL. Already during her Ph.D. studies, she joined Dr. George Calin's laboratory at M.D. Anderson Cancer Center in Houston, U.S.A. and continued as a post-doctoral fellow researching in depth the mechanisms of action of long and short non-coding RNAs in oncology.
In 2016, Dr Redis joined ProQR Therapeutics N.V in Leiden, NL as a scientist. ProQR is a biotech company focused on development of drugs to treat severe genetic disorders. Today, she is a senior scientist working on the development of a novel proprietary RNA editing technology - Axiomer® technology.

Dr. Amotz Shemi, IS
CEO
Silenseed

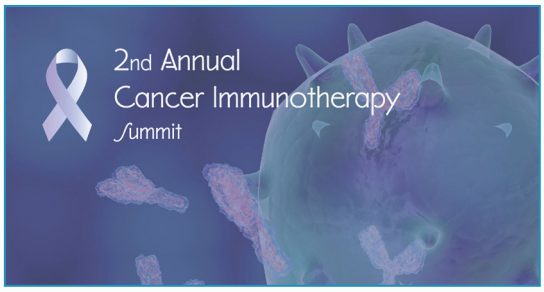
Dr. Amotz Shemi is the chief executive officer and a co-founder in Silenseed. Prior to Silenseed, Dr. Shemi served as senior VP technologies in Medinol LTD, a leading medical-stent company; and beforehand as the CEO of Color Chip, a leader in Ion-exchange based planar lightwave Circuits (PLC). Dr. Shemi brings with him 25 years of experience in end-to-end management from concept level via development, regulatory approvals to actual sales. Shemi received his Ph.D. degree in physics and astrophysics from the Tel Aviv University in Israel. Dr. Shemi is a lead inventor of more than dozen patents in the RNAi-delivery field, and an author and co-author of about 40 scientific papers.

Dr. Guillaume Pavlovic, FR
Department Head - Genetic Engineering & Model Validation Department
Phenomin - ICS
(former Institut Clinique de la Souris)

Dr. Guillaume Pavlovic is the head of the genetic engineering and model validation department at PHENOMIN - ICS. He is in charge of the generation and validation of genetically modified mouse and rat models for academic laboratories, European consortia and pharmaceutical or biotech companies. He leads R&D programmes focusing on the development of new tools for genetic engineering and microbiota analyses, and is an expert in genetics and genome editing including the CRISPR/Cas system.
After a Ph.D. in 2004, about bacterial mobile elements evolution, Guillaume Pavlovic worked at genOway, one of the lead companies in the generation of customised and ready-to-use genetically modified mouse and rat models to develop the molecular biology activity. He joined PHENOMIN - ICS (former Institut Clinique de la Souris) in 2007, as project manager in charge of the mouse model projects of its main industrial customers and was appointed at his present position in 2008.

Our Upcoming Events:

2nd Annual Cancer Immunotherapy Summit
21 - 22 September, 2017 | Vienna, Austria



Aseptic Processing Summit
24 - 25 October, 2017 | Berlin, Germany