SESSIONS

OPTIONAL PRE-CONFERENCE WORKSHOPS - 12/11/2019

Workshop Moderator's Opening Remarks

08:00 - 08:15
Workshop 2: Analytical Strategies and Technologies for Peptide Therapeutics

Participants

Vivian Lindo - Associate Director, Analytical Sciences, AstraZeneca

Analytical Characterization Tools for Peptide Therapeutics Physical Stability

08:15 - 08:45
Workshop 2: Analytical Strategies and Technologies for Peptide Therapeutics

Participants

Ana Santos - Principal Scientist, Formulations, Principal Scientist, Formulations

Managing CMC Activities to Accelerate Oligonucleotide Development and Manufacturing

08:30 - 09:45
Workshop 1: Managing CMC Activities to Accelerate Oligonucleotide Development and Manufacturing

Workshop Description:

This half-day pore-conference workshop will address early to mid-phase drug development and related CMC for oligonucleotide therapeutics. A detailed discussion on oligonucleotide therapeutics moving from discovery to clinical trials will be presented in form of case studies. This includes strategies for early clinical CMC development; early phase CMO work; GMP manufacturing; and the regulatory framework around these activities. Workshop attendees will be allowed 15 minutes open discussion after each presentation to deepen or clarify the presentations.

Who should attend?

Anyone interested in early to mid-stage development of oligonucleotide therapeutics; Anyone interested in outsourcing the manufacturing of oligonucleotide therapeutics to a CMO / CRO. This includes R&D Researchers, Manufacturing Personnel, Quality Assurance, Project Management, Business Development and Scientific Management.

Participants

Workshop Moderator:: Thomas Rupp - Owner & Principal, Thomas Rupp Consulting, Germany

Development and Validation of a Peptide Bioassay

08:45 - 09:15
Workshop 2: Analytical Strategies and Technologies for Peptide Therapeutics

Developed and validated biological assays provide a robust identity or meaningful functional potency measure, and are increasingly requested for synthetic peptides. Biological assays must be fit for a specific target, although many such assays have some common ground, such as statistical rigor to mitigate the inherent variability of biological systems. This presentation discusses our experience with biological assays as GMP release tests, using a GLP-1 peptide agonist as an example.

Participants

Michael Postlethwaithe, Ph.D - Business Development Manager, Bachem AG

Understanding the 3-D structures of a Peptide to Determine the Control Strategy for Biological Activity

09:15 - 09:45
Workshop 2: Analytical Strategies and Technologies for Peptide Therapeutics

A variety of analytical techniques were employed to gain insight to the higher order structures of a synthetic peptide. Based on the understanding gained, regulatory insight and bioassay stability data, no biological activity test was deemed necessary on the drug product specification. This position has been approved by regulatory authorities.

Participants

Mark Drew - Business Programme Lead, AstraZeneca

Networking Refreshment Break

09:45 - 10:15

Managing CMC Activities to Accelerate Oligonucleotide Development and Manufacturing

10:15 - 12:00
Workshop 1: Managing CMC Activities to Accelerate Oligonucleotide Development and Manufacturing

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Participants

Thomas Rupp - Owner & Principal, Thomas Rupp Consulting, Germany

Peptide Oligomers – Friends or Enemies?

10:15 - 10:45
Workshop 2: Analytical Strategies and Technologies for Peptide Therapeutics

Peptides offer enormous growth potential as future therapeutics and are recognized as being highly selective and efficacious. Due to their size, they generally have flexible structures and many have a preference for self-assembly. We will present how to investigate peptide structures in liquid formulations, with a focus on the ability to self-assemble as both stable structures and undesired higher order aggregates.

Participants

Lise Giehm, Ph.D - Principal Scientist, Zealand Pharma A/S

Late Breaking Presentation

10:45 - 11:15
Workshop 2: Analytical Strategies and Technologies for Peptide Therapeutics

Panel Discussion with Workshop Speakers

11:15 - 12:00
Workshop 2: Analytical Strategies and Technologies for Peptide Therapeutics

SESSIONS

OPTIONAL PRE-CONFERENCE WORKSHOPS - 12/11/2019

Close of Workshop

12:00 - 12:05

Workshop Moderator's Opening Remarks

13:00 - 13:15
Workshop 3: Drug Product Development Strategies for Oligonucleotides and Peptides

Participants

Stefan Vonhoff - Vice President CMC, NOXXON Pharma AG

Workshop Moderator's Opening Remarks

13:00 - 13:15
Workshop 4: Accelerating Oligonucleotide and Peptide Drug Development

Participants

Mimoun Ayoub, PhD - Director and Head of North American and Emerging Markets, CordenPharma International

Optimization of Novel Polymeric Delivery Vehicles by Chemical Evolution

13:15 - 13:45
Workshop 3: Drug Product Development Strategies for Oligonucleotides and Peptides

Chemical evolution for optimizing synthetic drug delivery carriers includes identification of delivery motifs (e.g. artificial amino acids), their assembly into defined sequences by solid phase synthesis, screening and selection for a defined cargo (nucleic acid, protein, Cas9/sgRNA) followed by carrier sequence variation and next selection round.

Participants

Ernst Wagner, Ph.D. - Professor and Chair, Pharmaceutical Biotechnology,, Ludwig Maximilans University

Stage Appropriate CMC Overview and Requirements for a Robust Dossier

13:15 - 13:45
Workshop 4: Accelerating Oligonucleotide and Peptide Drug Development

Participants

Mimoun Ayoub, PhD - Director and Head of North American and Emerging Markets, CordenPharma International

Investigations into Disruptive Delivery Approaches for LNA Antisense Oligonucleotides (ASO LNA)

13:45 - 14:15
Workshop 3: Drug Product Development Strategies for Oligonucleotides and Peptides

Parenteral or intrathecal administration of antisense oligonucleotides (ASO) have enabled treatment of liver and CNS based diseases, respectively, thanks to the inherent high exposure of the ASO in these tissues. To extend the possible scope of indications to treat with ASO, we looked at feasibility concepts to deliver ASO LNA into tissues where exposure with unformulated ASO LNA generally is insufficient for PD effect.

Participants

Dr. Michael Keller, Ph.D. - Senior Principal Scientist, pRED, pCMC, Roche

CMC Technical and Regulatory Strategies for Development of Peptides and Oligonucleotides

13:45 - 14:15
Workshop 4: Accelerating Oligonucleotide and Peptide Drug Development

Many factors guide the development pathways taken to meet drug demands and the regulatory requirements of a peptide or oligonucleotide. Key factors include: Finance, Support, Clinical Phase, Geography. I will overview the factors that influence CMC development of "tides" and strategies to keep the program successful when faced with obstacles.

Participants

Gary Musso, PhD - President, Musso and Associates LLC

Oligonucleotide Drug Product (Development) for (Ultra) Orphan Ophthalmic Diseases

14:15 - 14:45
Workshop 3: Drug Product Development Strategies for Oligonucleotides and Peptides

Oligonucleotide drug product (DP) development for (ultra) orphan ophthalmic diseases can be challenging from a formulation, primary packaging and manufacturing point of view. This presentation will elaborate on some of the challenges related to intravitreal (IVT) administered products, such as endotoxin and sub-visible particle specifications of DP. Also, considerations for dose accuracy, and the use of (prefilled) syringes will be discussed. Additionally, this presentation will include the possibility of terminal sterilization for oligonucleotide-based products.

Participants

Vera Brinks - Director, Pharmaceutics, ProQR Therapeutics

Scale-up Peptide Manufacturing Case Study: Transition from Solid-phase to Liquid Phase Synthesis

14:15 - 14:45
Workshop 4: Accelerating Oligonucleotide and Peptide Drug Development

Participants

Bruce Morimoto, PhD - Vice President, Drug Development Operations, Alkahest

Networking Refreshment Break

14:45 - 15:15

Challenges for Peptides Drug Products at the Interface of Formulation, Primary Packaging and Application

15:15 - 15:45
Workshop 3: Drug Product Development Strategies for Oligonucleotides and Peptides

Participants

Stephanie Lemoult, PhD - Senior Principal Scientist-Team Leader, Formulation, Lonza AG

Drug Product Development and Industrialization for Peptides and Oligos: A CDMO Perspective

15:15 - 15:45
Workshop 4: Accelerating Oligonucleotide and Peptide Drug Development

This presentation will provide a CDMO perspective on the challenges, the requirements and the technologies needed for the successful formulation, process development and industrialization of oligonucleotide and peptide-based drug products.

Participants

Umberto Romeo - R&D Manager, Corden Pharma

Formulation Development and Device Options for the Subcutaneous Injection of Spiegelmer Drug Product Solution

15:45 - 16:15
Workshop 3: Drug Product Development Strategies for Oligonucleotides and Peptides

For pegylated oligonucleotides, concentration and viscosity of the drug product solution are key parameters influencing the choice of devices suitable for subcutaneous self-administration. Data from a formulation development study aiming to reduce the viscosity of the pegylated Spiegelmer solution will be presented. Stability, compatibility and feasibility of the optimized drug product solution were evaluated in a range of devices. Conclusions for the further development of the drug product/device combination will be discussed.

Participants

Stefan Vonhoff - Vice President CMC, NOXXON Pharma AG

Late Breaking Presentation

15:45 - 16:15
Workshop 4: Accelerating Oligonucleotide and Peptide Drug Development

Late Breaking Presentation

16:15 - 16:45
Workshop 3: Drug Product Development Strategies for Oligonucleotides and Peptides

Panel Discussion with Workshop Speakers

16:15 - 17:00
Workshop 4: Accelerating Oligonucleotide and Peptide Drug Development

Concluding Remarks and Discussion

16:45 - 17:00
Workshop 3: Drug Product Development Strategies for Oligonucleotides and Peptides

Close of Workshops

17:00 - 17:05

SCHEDULE

OPTIONAL PRE-CONFERENCE WORKSHOPS - 12/11/2019

TIDES Europe: Oligonucleotide and Peptide Therapeutics

12-15 November 2019
RAI Amsterdam
Amsterdam, Netherlands

TIME	WORKSHOP 1: MANAGING CMC ACTIVITIES TO ACCELERATE OLIGONUCLEOTIDE DEVELOPMENT AND MANUFACTURING	WORKSHOP 2: ANALYTICAL STRATEGIES AND TECHNOLOGIES FOR PEPTIDE THERAPEUTICS	WORKSHOP 3: DRUG PRODUCT DEVELOPMENT STRATEGIES FOR OLIGONUCLEOTIDES AND PEPTIDES	WORKSHOP 4: ACCELERATING OLIGONUCLEOTIDE AND PEPTIDE DRUG DEVELOPMENT
08:00	**08:30** - Managing CMC Activities to Accelerate Oligonucleotide Development and Manufacturing	**08:00** - Workshop Moderator's Opening Remarks **08:15** - Analytical Characterization Tools for Peptide Therapeutics Physical Stability **08:45** - Development and Validation of a Peptide Bioassay		
09:00	**09:45** - Networking Refreshment Break	**09:15** - Understanding the 3-D structures of a Peptide to Determine the Control Strategy for Biological Activity **09:45** - Networking Refreshment Break	**09:45** - Networking Refreshment Break	**09:45** - Networking Refreshment Break
10:00	**10:15** - Managing CMC Activities to Accelerate Oligonucleotide Development and Manufacturing	**10:15** - Peptide Oligomers – Friends or Enemies? **10:45** - Late Breaking Presentation		
11:00		**11:15** - Panel Discussion with Workshop Speakers		
12:00	**12:00** - Close of Workshop	**12:00** - Close of Workshop	**12:00** - Close of Workshop	**12:00** - Close of Workshop
13:00			**13:00** - Workshop Moderator's Opening Remarks **13:15** - Optimization of Novel Polymeric Delivery Vehicles by Chemical Evolution **13:45** - Investigations into Disruptive Delivery Approaches for LNA Antisense Oligonucleotides (ASO LNA)	**13:00** - Workshop Moderator's Opening Remarks **13:15** - Stage Appropriate CMC Overview and Requirements for a Robust Dossier **13:45** - CMC Technical and Regulatory Strategies for Development of Peptides and Oligonucleotides

SCHEDULE

OPTIONAL PRE-CONFERENCE WORKSHOPS - 12/11/2019

TIDES Europe: Oligonucleotide and Peptide Therapeutics

12-15 November 2019
RAI Amsterdam
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TIME	WORKSHOP 1: MANAGING CMC ACTIVITIES TO ACCELERATE OLIGONUCLEOTIDE DEVELOPMENT AND MANUFACTURING	WORKSHOP 2: ANALYTICAL STRATEGIES AND TECHNOLOGIES FOR PEPTIDE THERAPEUTICS	WORKSHOP 3: DRUG PRODUCT DEVELOPMENT STRATEGIES FOR OLIGONUCLEOTIDES AND PEPTIDES	WORKSHOP 4: ACCELERATING OLIGONUCLEOTIDE AND PEPTIDE DRUG DEVELOPMENT
14:00	**14:45** - Networking Refreshment Break	**14:45** - Networking Refreshment Break	**14:15** - Oligonucleotide Drug Product (Development) for (Ultra) Orphan Ophthalmic Diseases **14:45** - Networking Refreshment Break	**14:15** - Scale-up Peptide Manufacturing Case Study: Transition from Solid-phase to Liquid Phase Synthesis **14:45** - Networking Refreshment Break
15:00			**15:15** - Challenges for Peptides Drug Products at the Interface of Formulation, Primary Packaging and Application **15:45** - Formulation Development and Device Options for the Subcutaneous Injection of Spiegelmer Drug Product Solution	**15:15** - Drug Product Development and Industrialization for Peptides and Oligos: A CDMO Perspective **15:45** - Late Breaking Presentation
16:00			**16:15** - Late Breaking Presentation **16:45** - Concluding Remarks and Discussion	**16:15** - Panel Discussion with Workshop Speakers
17:00	**17:00** - Close of Workshops	**17:00** - Close of Workshops	**17:00** - Close of Workshops	**17:00** - Close of Workshops

SESSIONS

MAIN CONFERENCE DAY 1 - 13/11/2019

Exhibit Viewing and Coffee in Poster and Exhibit Hall

07:30 - 08:10

Chairperson's Remarks

08:10 - 08:15
Keynote/Plenary Session

Opening the Central Nervous System for RNAi-based Modulation

08:15 - 08:50
Keynote/Plenary Session

RNAi enables simple and specific modulation of gene expression when the chemical architecture supporting efficient in vivo delivery is defined. Using huntingtin – the causative gene in Huntington's Disease – as a model, we demonstrate that chemically engineered siRNAs induce potent protein silencing (> 99%) in all brain regions tested one month post injection. Silencing persists for at least six months with the degree of gene modulation correlating to the level of the guide strand tissue accumulation. Opening the central nervous system for RNAi-based modulation of gene expression establishes a path toward the development of new cures for genetically defined neurodegenerative disorders.

Participants

Anastasia Khvorova, PhD - Professor, RNA Therapeutics Institute and Program in Molecular Medicine, University of Massachusetts Medical School

Linkers for Peptide Conjugation

08:50 - 09:25
Keynote/Plenary Session

Conjugation, understood as the linking of two moieties, which may be in the same or in a different molecule, is an effective chemistry approach to create new entities with synergistic properties. The key factor is the linker. Herein, we will present several linkers useful for the preparation of cyclic and branch peptides and peptide and antibody drug conjugates as well.

Participants

Fernando Albericio, PhD - Department of Organic Chemistry, University of Barcelona

Networking Refreshment Break in Poster and Exhibit Hall

09:25 - 10:10
Keynote/Plenary Session

mRNA Vaccines and Therapeutics: From Promise to Reality

10:10 - 10:45
Keynote/Plenary Session

Participants

Hari Pujar, PhD - Vice President, Technical Development and Manufacturing, Moderna Therapeutics

Development of Delivery Systems for Biopharmaceuticals within the IMI COMPACT Consortium: Results and Lessons Learned

10:45 - 11:20
Keynote/Plenary Session

COMPACT, an IMI sponsored public-private partnership between 23 academic groups, SMEs and pharmaceutical companies has collectively worked on the delivery issues of biopharmaceuticals in the period 2012-2017. Besides making and testing novel drug delivery systems optimized to meet the demands for crossing specific biological barriers (e.g. blood-brain barrier, intestinal barrier, air-to-lung barrier as well as intracellular barriers), new tools and complex in vitro models were developed. In this presentation, I will highlight some of the achievements and discuss lessons learned after 5 years COMPACT.

Participants

Enrico Mastrobattista, PhD - Professor of Pharmaceutical Biotechnology and Delivery, Utrecht University

Antisense for a Billion People: The Development of RNA-based Therapy for Elevated Lipoprotein(a)

11:20 - 11:55
Keynote/Plenary Session

Elevated Lp(a) (>50 mg/dL or >125 nmol/L) is estimated to be present in 1.4 billion people. Elevated Lp(a) is associated with cardiovascular disease and aortic stenosis. Due to its high plasma levels and hepatocyte origin, Lp(a) can only be effectively targeted by inhibiting its synthesis using RNA therapeutics. The development of an antisense strategy in reducing Lp(a) plasma levels will be reviewed from pre-clinical models to phase 3 clinical trials powered to evaluate cardiovascular event reduction.

Participants

Sotirios Tsimikas, M.D. - Vice President of Global Cardiovascular Development, Ionis Pharmaceuticals

Transition to Spotlight Presentation Rooms

11:55 - 12:00
Keynote/Plenary Session

Guiding RNA Formulations from Laboratory into Clinical Trials. Lessons Learned from Development and Optimization of Liposomal Formulations

12:00 - 12:30
Sponsored Spotlight Presentation 1

Over the past few years liposomal drug preparations have been increasingly used in clinical trials. Until now, several liposomal products have reached the market, many other formulations are still in the pipeline. For all these products, simple, economic and GMP-conform production techniques and facilities are necessary. Here, several points to consider already at the stage of process and product transfer to the CMO should be listed. Product development at early stage should implement the use of high-quality raw materials, robust and stable product and process conditions and robust analytical methods. The whole system should be implemented in a robust QA system. Furthermore, the production system should be designed to allow scalable and sterile manufacturing. In addition, it should meet several requirements, such as simplicity, robustness and easy handling of sterilisation procedures. Furthermore, the modified ethanol injection technique itself is distinguished by mild preparation conditions and the avoidance of hazardous solvents and forces, which may disrupt lipids as well as entrapped substances. Data will be presented, which describe impact of process conditions on the generated particle size and homogeneity. A few examples of drug products and related processes will be shown, where special focus will be set on influencing particle size and size distribution by varying the process parameters of the Polymun liposome technology.

Participants

Andreas Wagner - Head of Liposome Technology, Polymun Scientific GmbH

ZEOsphere DRP Mixed-Mode for Oligonucleotide Purification

12:00 - 12:30
Sponsored Spotlight Presentation 2

Participants

Victoria Custodis - Team Leader R&D, Zeochem AG

Intertek Briefing

12:00 - 12:30
Sponsored Spotlight Presentation 3

SESSIONS

MAIN CONFERENCE DAY 1 - 13/11/2019

A Single-Use Solution to Bulk Tray Lyophilization of Polypeptide and Oligonucleotide Therapeutics

12:00 - 12:30
Sponsored Spotlight Presentation 4

A recent survey of CMOs specializing in the synthesis and purification of polypeptide and oligonucleotide therapeutics indicates that operator safety and elimination of stainless steel tray cleaning are priorities for these manufacturers. These needs, as well as others, will be discussed within the context of bulk tray lyophilization. Information on a disposable single-use lyophilization tray, which provides both isolation and containment of the contents in the tray, will be shared.

Participants

Scott Ross - Global Product Specialist, W.L. Gore & Associates

Networking Luncheon in Poster and Exhibit Hall

12:30 - 13:50

Chairman's Remarks

13:50 - 14:00
Oligonucleotide Discovery, Preclinical and Clinical

Participants

Troels Koch, PhD - Vice President and Head of Research, RNA Therapeutics, Roche pRED, Roche Innovation Center Copenhagen

Chairperson's Remarks

13:50 - 14:00
Oligonucleotide Chemistry, Manufacturing and Controls

Participants

Nadim Akhtar, PhD - Principal Scientist, AstraZeneca

Chairperson's Remarks

13:50 - 14:00
Peptide Discovery, Preclinical and Clinical

Participants

Rami Hannoush, PhD - Principal Scientist & Group Leader, Genentech

Chairman's Remarks

13:50 - 14:00
Peptide Chemistry, Manufacturing and Controls

Participants

Neil Thompson - Senior Director, Business Development Europe, PolyPeptide Group

Machine Learning-guided Design of Antisense Oligonucleotides

14:00 - 14:30
Oligonucleotide Discovery, Preclinical and Clinical

Antisense oligonucleotides are particularly suited for machine learning-guided drug design. As oligomers, they can be easily represented digitally, and any predicted sequence is straightforward to synthesize using standard phosphoramidite building blocks. Recent examples, enabled by careful organization and labeling of preclinical datasets across multiple discovery projects, will be presented.

Participants

Peter Hagedorn - Senior Principal Scientist, Group Leader, Roche Innovation Center Copenhagen A/S

Characterization of Raw Materials for the Manufacturing of Oligonucleotides

14:00 - 14:30
Oligonucleotide Chemistry, Manufacturing and Controls

Raw materials are key sources for a number of impurites found in oligonucleotides. Therefore, the control of raw materials and especially of potential reactive or critial by-products are mandatory to obtain a high level of batch to batch reproducibility. This presentation will summarize general analytical methods for the characterization and control of raw materials. Moreover, it will focus on some reactive components found in phosphoramidites as well as in some other raw materials.

Participants

Huseyin Ayguen - Chief Scientific Officer, BioSpring

Early Implementation of Appropriate Studies to Identify Preclinical Liabilities is Key to Success in Peptide Drug Discovery

14:00 - 14:30
Peptide Discovery, Preclinical and Clinical

An appropriate screening strategy was implemented to support peptide drug discovery programs. This presentation will cover valuable studies necessary to address specific issues in peptide development such as sub cutaneous and plasma metabolism with in vitro/in vivo correlation to optimize bioavailability; potential peptide-induced pseudo-allergic reactions; immunogenicity and aggregation/oligomerization tendencies.

Participants

Federica Orvieto - Senior Research Investigator, Peptide Chemistry, IRBM Science Park SPA

Gly-His Tag Acylation for N-terminal Chemical Modification of Proteins

14:00 - 14:30
Peptide Chemistry, Manufacturing and Controls

Site-selective modification of proteins is highly desirable for the controlled introduction of small probes like biotin or larger moieties such as PEG. We recently reported the development of a new His tag, Gly-His3-6, for highly selective N-terminal acylation. New extensions of this method are presented. Finally, a new linker strategy for the synthesis of C-terminally peptides and the use of automated high-performance flash chromatography for the purification of the peptides in this project will be discussed.

Participants

Knud Jensen, Ph.D - Professor, Department of Chemistry, University of Copenhagen

Novel Chemistries for RNAi Therapeutics

14:30 - 15:00
Oligonucleotide Discovery, Preclinical and Clinical

Participants

Muthiah (Mano) Manoharan, PhD - Senior Vice President of Drug Discovery, Alnylam Pharmaceuticals, Inc.

Purge-based Risk Assessment for Solvent and Small Molecule Impurities Generated during Oligonucleotide Manufacture

14:30 - 15:00
Oligonucleotide Chemistry, Manufacturing and Controls

Through the European Pharmaceutical Oligonucleotide Consortium, a team of companies is exploring opportunities and generating supporting data to justify the exclusion of small molecule impurities and solvents from release testing through the use of risk based purge arguments. To support the theoretical purge arguments, spike and purge studies have been performed in different processes and by multiple companies.

Participants

Ben Andrews, Ph.D. - Scientific Investigator, GlaxoSmithKline

SESSIONS

MAIN CONFERENCE DAY 1 - 13/11/2019

Developability and Preformulation of Peptides

14:30 - 15:00
Peptide Discovery, Preclinical and Clinical

When new peptide drug candidates are identified, it is key to assess their suitability to be successfully developed as stable drug products for the intended route of administration. An approach to assess and compare several peptide drug candidates prior to entering Development from Research will be presented and examples given.

Participants

Jette Boll - Senior Research Scientist, Ferring Pharmaceuticals

Improving Peptide Manufacturing and Process Performance

14:30 - 15:00
Peptide Chemistry, Manufacturing and Controls

Over the last few years, a multidisciplinary organization has been set up to better understand and control peptide synthesis, merging the skills of experienced peptide chemists with the tools of chemical engineers and the devices of advanced control experts. These tools are combining decades of experience with a fundamental study of solid phase chemistry (modelling approach). The outcome of this study is now put into practice and is combined with Advanced Control Technologies, to offer a new way of manufacturing peptides.

Participants

Olivier Ludemann-Hombourger, PhD - Global Director Innovation and Strategy, PolyPeptide Group

Gap Modifications Improve Therapeutic Index of Gapmer ASOs

15:00 - 15:30
Oligonucleotide Discovery, Preclinical and Clinical

Introducing chemical modifications in the DNA gap-region can enhance the therapeutic profile of gapmer ASOs. Results from our comprehensive structure activity relationships for evaluating gap-modifications including controlling PS-chirality will be presented.

Participants

Michael Oestergaard - Research Fellow, Ionis Pharmaceuticals

Deeper Understanding of Separation of Native and Phosphorothioated Oligonucleotides and Their Impurities Using Ion-pair Reversed Phase Chromatography

15:00 - 15:30
Oligonucleotide Chemistry, Manufacturing and Controls

Separation of oligonucleotides were fundamentally studied. Diastereomer separation was controlled by choosing the right ion-pairing reagent and stationary phase. Phosphorothioated oligonucleotides could be purified at high purity and yield using appropriate conditions, due to the displacement of impurities. Here, the phenyl column showed better results compared to the alkyl columns.

Participants

Dr. Martin Enmark - Researcher, Karlstad University

Emerging Approaches in Peptide Drug Discovery and Their Applications in Targeting Protein-Protein Interactions

15:00 - 15:30
Peptide Discovery, Preclinical and Clinical

This talk will describe our group's efforts in discovering and optimizing peptide-based scaffolds and will highlight some of the challenges and novel technologies for peptide lead identification and development. A case study on a peptide antagonist with a unique mode of inhibition will be discussed.

Participants

Rami Hannoush, PhD - Principal Scientist & Group Leader, Genentech

Ultra-fast Development and Optimization of Large-Scale Peptide Manufacturing Processes

15:00 - 15:30
Peptide Chemistry, Manufacturing and Controls

This presentation will discuss data related to how we develop a manufacturing process in large scale by adapting a continuous approach and implementation of DoE and QbD enabling multivariate optimizations in a single manufacturing run. It will describe how we run 30 large scale peptide manufacturing development runs in 4 weeks. The presentation will contain lots of novel experimental data with high scientific and regulatory impact, none of which has been presented before.

Participants

Jens Bukrinski - Head of R&D, SB3000 Ltd.

Networking Refreshment Break in Poster and Exhibit Hall

15:30 - 16:00

Control of Backbone Stereochemistry Provides a New Dimension for the Optimization of Oligonucleotide Drug Candidates

16:00 - 16:30
Oligonucleotide Discovery, Preclinical and Clinical

Participants

Dr. Meiling Li - Scientist, Hoffmann-La Roche

Strategies for Identity Testing of Oligonucleotide Therapeutics

16:00 - 16:30
Oligonucleotide Chemistry, Manufacturing and Controls

Due to their large size and polymeric nature, establishing identity of oligonucleotide therapeutics is significantly more challenging compared to synthetic small molecules. Techniques such as molecular weight conformation and retention time matching that are commonly employed and readily accepted for small molecules are generally deemed insufficient for identity confirmation of the oligonucleotide. This presentation will discuss various risk factors during manufacturing process that can potentially lead to an oligonucleotide of an incorrect structure, along with currently available analytical methods that can detect different structural changes. A risk-based framework for the selection of identity tests and how it can be integrated into a robust control strategy will be proposed.

Participants

Nadim Akhtar, PhD - Principal Scientist, AstraZeneca

Development of the Stable, Fast Acting Glucagon Analogue NN9513 for Clinical Testing

16:00 - 16:30
Peptide Discovery, Preclinical and Clinical

The stable glucagon analogue NN9513 to be used in a prefilled ready-to-use device was developed. Native glucagon has poor physical and chemical stability so major improvements of stability were required. Introduction of glutamic acid moieties on the sidechain of position 24 increased physical stability dramatically. Several amino acid substitutions were required to achieve the required chemical stability. In vitro and preclinical in vivo PK and PD data will also be presented.

Participants

Jesper F. Lau, PhD - Scientific Director, Research Chemistry, Novo Nordisk A/S

SESSIONS

MAIN CONFERENCE DAY 1 - 13/11/2019

Development of New Multicolumn Processes and the Presentation of a New Concept with a Single Column

16:00 - 16:30
Peptide Chemistry, Manufacturing and Controls

Participants

Jose Paolo Mota - Professor, Chemical & Biochemical Engineering, Universidade NOVA de Lisboa

Introduction of Non-chiral Phosphorodithioates into Locked Nucleic Acids

16:30 - 17:00
Oligonucleotide Discovery, Preclinical and Clinical

With the recent launches of chemically modified oligonucleotides, RNA therapeutics have clearly demonstrated their medical benefit. Particularly phosphorothioates have been extensively profiled and cover most of the clinically investigated entities. However the complexity of diastereoisomers have resulted in the great challenges in understanding the PK/PD of stereomixed oligonucleotides. Introduction of non-chiral phosphorodithioates dramatically reduces the diastereomeric complexity. As we are constantly developing our LNA platform, potential next generation analogues have been identified, showing very promising drug properties. Here we will report our latest observations of backbone modified Locked Nucleic Acids with a particular focus on non-chiral phosphorodithioate modifications, including stereo-defined internucleoside linkages. Several strategies how to rapidly identify highly potent one single phosphorothioate LNA isomer will be discussed and medicinal chemistry aspects highlighted, supported by recent in vitro and in vivo data.

Participants

Dr. Chandra Vargeese, PhD - SVP, Head of Drug Discovery, WAVE Life Sciences

Phase Appropriate Method Validation Strategies for Antisense Oligonucleotides with Accelerated Product Development Timelines

16:30 - 17:00
Oligonucleotide Chemistry, Manufacturing and Controls

Antisense oligonucleotides (ASOs) often target rare disease indications with unmet medical need leading to expedited product development timelines. Balancing clinical phase-appropriate practices with the requirements of readiness for commercial licensure can be challenging. An overall strategy for managing analytical method validation over the product development lifecycle from R2D to commercial is proposed. Case studies for phase appropriate analytical method validation of expedited programs will be presented.

Participants

Stacey Traviglia, Ph.D. - Associate Director, QC Analytical Technology, Biogen

Outer Membrane Targeting Antibiotics (OMPTA): Preclinical and Clinical Development of a Novel Class of Antibiotics against Life-threatening Gram-negative Infections

16:30 - 17:00
Peptide Discovery, Preclinical and Clinical

The presentation will focus on the discovery and development of the OMPTA class of antibiotics to treat life-threatening Gram-negative infections. Murepavadin has entered phase III trials and is the first representative of the OMPTA class, whereas preclinical stage POL7306 is a medium-spectrum antibiotic with potent activity against all WHO priority 1 Gram-negative pathogens including MDR, XDR, and colistin-resistant pathogens.

Participants

Dr. Anatol Luther - Head of Chemistry, Polyphor Ltd

Continuous Chromatography of Synthetic Peptides

16:30 - 17:00
Peptide Chemistry, Manufacturing and Controls

The current modus operandi for the downstream processing of synthetic peptides is reversed phase chromatography performed in batch mode. Multicolumn Countercurrent Solvent Gradient Purification (MCSGP) is expected to be a disruptive technology for this very cost intensive part of synthetic peptide manufacturing. Results from continuous peptide purifications will be presented and compared to traditional batch chromatography. Potential implementation strategies for this technology will be discussed.

Participants

Ralf Eisenhuth, PhD - Process Manager Technology Transfer and Chromatography, Bachem AG

Secarna's LNAplusTM ASOs for Treatment of Cancer and Kidney Disease

17:00 - 17:30
Oligonucleotide Discovery, Preclinical and Clinical

Secarna has developed a proprietary platform to identify highly active and well-tolerated LNA-modified antisense oligonucleotides. Preclinical data will be presented showing antitumor activity of ASOs targeting the immunosuppressive tumor microenvironment. Furthermore, efficacy of ASOs targeting an endoplasmatic reticulum stress factor is shown in an in vivo model of diabetic nephropathy.

Participants

Dr. Frank Jaschinski - CSO, Secarna Pharmaceuticals

Panel Discussion with Session Speakers

17:00 - 17:30
Oligonucleotide Chemistry, Manufacturing and Controls

Participants

Moderator:: Nadim Akhtar, PhD - Principal Scientist, AstraZeneca

Development of Novel Peptide Therapeutics

17:00 - 17:30
Peptide Discovery, Preclinical and Clinical

Participants

Efrat Halbfinger, PhD - Senior Director of Chemistry, BioLineRx Ltd.

Panel Discussion with Session Speakers

17:00 - 18:00
Peptide Chemistry, Manufacturing and Controls

Participants

Neil Thompson - Senior Director, Business Development Europe, PolyPeptide Group

Close of Sessions

18:00 - 18:05

Attendee Networking Reception Event at The Boat House at Strandzuid

18:05 - 19:35

SCHEDULE

MAIN CONFERENCE DAY 1 - 13/11/2019

TIDES Europe: Oligonucleotide and Peptide Therapeutics

12-15 November 2019
RAI Amsterdam
Amsterdam, Netherlands

TIME	KEYNOTE/PLENARY SESSION	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	PEPTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY, PRECLINICAL AND CLINICAL	SPONSORED SPOTLIGHT PRESENTATION 1	SPONSORED SPOTLIGHT PRESENTATION 2	SPONSORED SPOTLIGHT PRESENTATION 3	SPONSORED SPOTLIGHT PRESENTATION 4
07:00	**07:30** - Exhibit Viewing and Coffee in Poster and Exhibit Hall	**07:30** - Exhibit Viewing and Coffee in Poster and Exhibit Hall	**07:30** - Exhibit Viewing and Coffee in Poster and Exhibit Hall	**07:30** - Exhibit Viewing and Coffee in Poster and Exhibit Hall	**07:30** - Exhibit Viewing and Coffee in Poster and Exhibit Hall	**07:30** - Exhibit Viewing and Coffee in Poster and Exhibit Hall	**07:30** - Exhibit Viewing and Coffee in Poster and Exhibit Hall	**07:30** - Exhibit Viewing and Coffee in Poster and Exhibit Hall	**07:30** - Exhibit Viewing and Coffee in Poster and Exhibit Hall
08:00	**08:10** - Chairperson's Remarks **08:15** - Opening the Central Nervous System for RNAi-based Modulation **08:50** - Linkers for Peptide Conjugation								
09:00	**09:25** - Networking Refreshment Break in Poster and Exhibit Hall								

SCHEDULE

MAIN CONFERENCE DAY 1 - 13/11/2019

TIME	KEYNOTE/PLENARY SESSION	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	PEPTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY, PRECLINICAL AND CLINICAL	SPONSORED SPOTLIGHT PRESENTATION 1	SPONSORED SPOTLIGHT PRESENTATION 2	SPONSORED SPOTLIGHT PRESENTATION 3	SPONSORED SPOTLIGHT PRESENTATION 4
10:00	**10:10** - mRNA Vaccines and Therapeutics: From Promise to Reality **10:45** - Development of Delivery Systems for Biopharmaceuticals within the IMI COMPACT Consortium: Results and Lessons Learned								
11:00	**11:20** - Antisense for a Billion People: The Development of RNA-based Therapy for Elevated Lipoprotein(a) **11:55** - Transition to Spotlight Presentation Rooms								

SCHEDULE

MAIN CONFERENCE DAY 1 - 13/11/2019

TIME	KEYNOTE/PLENARY SESSION	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	PEPTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY, PRECLINICAL AND CLINICAL	SPONSORED SPOTLIGHT PRESENTATION 1	SPONSORED SPOTLIGHT PRESENTATION 2	SPONSORED SPOTLIGHT PRESENTATION 3	SPONSORED SPOTLIGHT PRESENTATION 4
12:00	**12:30** - Networking Luncheon in Poster and Exhibit Hall	**12:30** - Networking Luncheon in Poster and Exhibit Hall	**12:30** - Networking Luncheon in Poster and Exhibit Hall	**12:30** - Networking Luncheon in Poster and Exhibit Hall	**12:30** - Networking Luncheon in Poster and Exhibit Hall	**12:00** - Guiding RNA Formulations from Laboratory into Clinical Trials. Lessons Learned from Development and Optimization of Liposomal Formulations **12:30** - Networking Luncheon in Poster and Exhibit Hall	**12:00** - ZEOsphere DRP Mixed-Mode for Oligonucleotide Purification **12:30** - Networking Luncheon in Poster and Exhibit Hall	**12:00** - Intertek Briefing **12:30** - Networking Luncheon in Poster and Exhibit Hall	**12:00** - A Single-Use Solution to Bulk Tray Lyophilization of Polypeptide and Oligonucleotide Therapeutics **12:30** - Networking Luncheon in Poster and Exhibit Hall
13:00		**13:50** - Chairperson's Remarks	**13:50** - Chairman's Remarks	**13:50** - Chairman's Remarks	**13:50** - Chairperson's Remarks				
14:00		**14:00** - Characterization of Raw Materials for the Manufacturing of Oligonucleotides **14:30** - Purge-based Risk Assessment for Solvent and Small Molecule Impurities Generated during Oligonucleotide Manufacture	**14:00** - Machine Learning-guided Design of Antisense Oligonucleotides **14:30** - Novel Chemistries for RNAi Therapeutics	**14:00** - Gly-His Tag Acylation for N-terminal Chemical Modification of Proteins **14:30** - Improving Peptide Manufacturing and Process Performance	**14:00** - Early Implementation of Appropriate Studies to Identify Preclinical Liabilities is Key to Success in Peptide Drug Discovery **14:30** - Developability and Preformulation of Peptides				

SCHEDULE

MAIN CONFERENCE DAY 1 - 13/11/2019

TIME	KEYNOTE/PLENARY SESSION	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	PEPTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY, PRECLINICAL AND CLINICAL	SPONSORED SPOTLIGHT PRESENTATION 1	SPONSORED SPOTLIGHT PRESENTATION 2	SPONSORED SPOTLIGHT PRESENTATION 3	SPONSORED SPOTLIGHT PRESENTATION 4
15:00	**15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:00** - Deeper Understanding of Separation of Native and Phosphorothioated Oligonucleotides and Their Impurities Using Ion-pair Reversed Phase Chromatography **15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:00** - Gap Modifications Improve Therapeutic Index of Gapmer ASOs **15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:00** - Ultra-fast Development and Optimization of Large-Scale Peptide Manufacturing Processes **15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:00** - Emerging Approaches in Peptide Drug Discovery and Their Applications in Targeting Protein-Protein Interactions **15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:30** - Networking Refreshment Break in Poster and Exhibit Hall	**15:30** - Networking Refreshment Break in Poster and Exhibit Hall
16:00		**16:00** - Strategies for Identity Testing of Oligonucleotide Therapeutics **16:30** - Phase Appropriate Method Validation Strategies for Antisense Oligonucleotides with Accelerated Product Development Timelines	**16:00** - Control of Backbone Stereochemistry Provides a New Dimension for the Optimization of Oligonucleotide Drug Candidates **16:30** - Introduction of Non-chiral Phosphorodithioates into Locked Nucleic Acids	**16:00** - Development of New Multicolumn Processes and the Presentation of a New Concept with a Single Column **16:30** - Continuous Chromatography of Synthetic Peptides	**16:00** - Development of the Stable, Fast Acting Glucagon Analogue NN9513 for Clinical Testing **16:30** - Outer Membrane Targeting Antibiotics (OMPTA): Preclinical and Clinical Development of a Novel Class of Antibiotics against Life-threatening Gram-negative Infections				

SCHEDULE

MAIN CONFERENCE DAY 1 - 13/11/2019

TIME	KEYNOTE/PLENARY SESSION	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	PEPTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY, PRECLINICAL AND CLINICAL	SPONSORED SPOTLIGHT PRESENTATION 1	SPONSORED SPOTLIGHT PRESENTATION 2	SPONSORED SPOTLIGHT PRESENTATION 3	SPONSORED SPOTLIGHT PRESENTATION 4
17:00		**17:00** - Panel Discussion with Session Speakers	**17:00** - Secarna's LNAplusTM ASOs for Treatment of Cancer and Kidney Disease	**17:00** - Panel Discussion with Session Speakers	**17:00** - Development of Novel Peptide Therapeutics				
18:00	**18:00** - Close of Sessions **18:05** - Attendee Networking Reception Event at The Boat House at Strandzuid	**18:00** - Close of Sessions **18:05** - Attendee Networking Reception Event at The Boat House at Strandzuid	**18:00** - Close of Sessions **18:05** - Attendee Networking Reception Event at The Boat House at Strandzuid	**18:00** - Close of Sessions **18:05** - Attendee Networking Reception Event at The Boat House at Strandzuid	**18:00** - Close of Sessions **18:05** - Attendee Networking Reception Event at The Boat House at Strandzuid	**18:00** - Close of Sessions **18:05** - Attendee Networking Reception Event at The Boat House at Strandzuid	**18:00** - Close of Sessions **18:05** - Attendee Networking Reception Event at The Boat House at Strandzuid	**18:00** - Close of Sessions **18:05** - Attendee Networking Reception Event at The Boat House at Strandzuid	**18:00** - Close of Sessions **18:05** - Attendee Networking Reception Event at The Boat House at Strandzuid

SESSIONS

MAIN CONFERENCE DAY 2 - 14/11/2019

Sponsored Breakfast Spotlight Presentation

08:00 - 08:40

Syngene Briefing

Chairperson's Remarks

08:40 - 08:45
Keynote/Plenary Session

Global Development Programme of RG6042, an Antisense Oligonucleotide, for the Treatment of Huntington's Disease

08:45 - 09:15
Keynote/Plenary Session

RG6042 is an antisense oligonucleotide in clinical development designed to lower HTT protein production by selectively targeting HTT mRNA. In a Phase I/IIa study, RG6042 safely lowered CSF mutant HTT (mHTT) in early Huntington's disease (HD), prompting Roche to begin a Global Development Programme (GDP). Roche's GDP will provide valuable information on the clinical benefit and safety of RG6042, as well as further longitudinal evidence of the causal role of mHTT in disease progression.

Participants

Scott Schobel - Associate Group Medical Director, F. Hoffman-La Roche Ltd.

Plants as Biofactories for Producing Peptide-based Pharmaceuticals

09:15 - 09:45
Keynote/Plenary Session

We are using crop plants as expression systems for the production of pharmaceutically active cyclic peptides. This presentation will give an overview on the biosynthesis and applications of cyclic peptides and describe the use of tobacco, Arabidopsis and petunia plants in as vehicles for the production of peptide-based drug leads for cancer, cardiovascular disease and pain.

Participants

David Craik, PhD - Professor of Biomolecular Structure, Institute for Molecular Bioscience, University of Queensland

Regulatory Quality and CMC Perspectives on mRNA Vaccines and Peptide Vaccine Adjuvants

09:45 - 10:15
Keynote/Plenary Session

mRNA vaccines are an ever-increasing new supplement to existing conventional vaccines. They are easy to manufacture, yet pose some CMC challenges regarding functional characterization and stability. As expressed proteins and peptides are frequently not sufficiently immunogenic by themselves, adjuvants are needed in their formulation. Hence, Major CMC aspects for novel adjuvants will be addressed.

Participants

Dr. Ralf Wagner - Head Section Viral Vaccines, Paul-Ehrlich-Institut

Networking Refreshment Break in Poster and Exhibit Hall

10:15 - 10:55

A Case Study of Concurrent Global Regulatory Filings for Two Oligonucleotides

10:55 - 11:25
Regulatory Strategies for Oligonucleotides and Peptides

A case study will be presented that compares the submitted module 3 content for two similar oligonucleotide drugs. Significant differences between the dossiers will be detailed in combination with regulatory agency feedback relevant to the differences. An evaluation of the success of each approach will be presented, and how learnings from the case study may inform future filing strategies.

Participants

Jennifer Franklin - Director, CMC Regulatory Affairs, Ionis Pharmaceuticals

Experience with Early and Late Phase Global Submissions of Oligonucleotide-based Products

11:25 - 11:55
Regulatory Strategies for Oligonucleotides and Peptides

Oligonucleotides are a relatively new class of drugs with the potential to treat a wide spectrum of indications with a wide variety of therapeutic approaches. The number of companies that included oligonucleotides into their portfolio significantly increased in the last years, as well as the number of approvals of therapeutic medicines containing oligonucleotides. Most of these oligonucleotide-based medicines are approved in the major markets (EU, US, JP). However, there is still limited experience in terms of global regulatory expectations for this type of products. We would like to present an overview of the major topics that were raised from various HAs during early phase and late phase submissions.

Participants

Cinzia Gazziola - Technical Regulatory Affairs Manager, Hoffmann-La Roche

Peptide Regulatory Strategies and Experiences

11:55 - 12:25
Regulatory Strategies for Oligonucleotides and Peptides

Speaker TBA

Participants

Peter Larsson - Global Director Regulatory Affairs, PolyPeptide Group

Transition to Spotlight Presentation Rooms

12:25 - 12:30
Regulatory Strategies for Oligonucleotides and Peptides

Mass-Production of Target RNA by Microorganism

12:30 - 13:00
Sponsored Spotlight Presentation 1

Recently, functional RNA and its application to nucleic acid-based drugs attract lots of attention. It is indispensable to produce the target RNA molecules of interest at low cost and in large-scale through biological production system. It seems that the conventional production of recombinant RNAs using mainly E. coli are not sufficient in the productivity and the stability of the production system that can cope with mass-production of recombinant RNA of interest. In this study, we have developed a fundamental system of efficient production for target RNA molecules in our microbial strain, Corynebacterium glutamicum. Using the system, we will present some successful instances of production of RNAs which could be active pharmaceutical ingredients for nucleic acid-drugs. Thus, our system will be able to serve as an efficient platform for preparation of RNAs of interest in large amounts.

Participants

Shuhei Hashiro - Research Scientist, Ajinomoto Co., Inc.

Quantification of Selected Impurities in Oligonucleotides

12:30 - 13:00
Sponsored Spotlight Presentation 2

The quantification of oligonucleotide related impurities is commonly performed based on UV260nm. In some cases, the separation of certain impurities by liquid chromatography is not possible or the specific impurity has no sufficient UV absorbance. To quantify and monitor such impurities, alternative procedures and detectors are needed. This presentation provides an overview of possible approaches to quantify oligonucleotide related impurities, focusing especially on but not limited to the quantification by mass spectrometry.

Participants

Huseyin Ayguen - Chief Scientific Officer, BioSpring

Precision Nanosystems Briefing

12:30 - 13:00
Sponsored Spotlight Presentation 3

Changing the Tide in Peptides - Peptide Purification and Modification with Beylntic's Catch-and-Release Technology

12:30 - 12:45
Sponsored Spotlight Presentation 4

Peptide purification along the pharmaceutical value chain becomes more and more challenging due to rising demands for difficult peptides, chemical peptide modification or high-throughput missions. Belyntic has taken that challenge and has developed Peptide Easy Clean (PEC), a novel Catch-and-Release technology for the parallel purification of chemically synthesized peptides. As a truly orthogonal method to chromatography, PEC represents a new purification tool for the difficult cases, while also allowing the concurrent introduction of chemical modifications to the peptides of interest.

Participants

Oliver Reimann, PhD - Co-Founder, Belyntic GmbH

Networking Luncheon in Poster and Exhibit Hall

13:00 - 14:25

Co-Chairpersons' Remarks

14:25 - 14:30
Oligonucleotide Discovery, Preclinical and Clinical

Participants

Muthiah (Mano) Manoharan, PhD - Senior Vice President of Drug Discovery, Alnylam Pharmaceuticals, Inc.

Dmitry Samarsky, PhD - Chief Technology Officer, Sirnaomics

Co-Chairpersons' Remarks

14:25 - 14:30
Oligonucleotide Chemistry, Manufacturing and Controls

Participants

Muthiah (Mano) Manoharan, PhD - Senior Vice President of Drug Discovery, Alnylam Pharmaceuticals, Inc.

Dmitry Samarsky, PhD - Chief Technology Officer, Sirnaomics

Chairperson's Remarks

14:25 - 14:30
Peptide Discovery, Preclinical and Clinical

Participants

Bruce Morimoto, PhD - Vice President, Drug Development Operations, Alkahest

Chairperson's Remarks

14:25 - 14:30
Peptide Chemistry, Manufacturing and Controls

Chairperson's Remarks

14:25 - 14:30
mRNA Therapeutics and CRISPR Therapeutics

Overcoming Extra- and Intracellular Barriers: Polymer-based mRNA Delivery Systems

14:30 - 15:00
Oligonucleotide Discovery, Preclinical and Clinical

Messenger RNA has long been considered too unstable to be a valuable tool for cell transfection. This has limited the interest in its application for a long time. In recent years, however, it has become clear that there are ways to cope with this instability by showing that complex formation with cationic lipids or polymers provides effective protection of mRNA against degradation. CureVac has developed and optimized its own versatile delivery platform (CureVac Carrier Molecule – CVCM), which can be tailored to deliver therapeutic mRNAs to different organs and tissues. We have established a broad range of in vitro and in vivo assays allowing the identification of suitable formulations for broad range of applications. Here we report on a panel of mRNA formulations, which were tested for their efficacy to transfect cells in the lung and the eye.

Participants

Dr. Joanna Rejman - Associate Director Neurologic and Pulmonary Diseases, CureVac AG

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Oligonucleotide Chemistry, Manufacturing and Controls

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Participants

Dr. Joanna Rejman - Associate Director Neurologic and Pulmonary Diseases, CureVac AG

SESSIONS

MAIN CONFERENCE DAY 2 - 14/11/2019

Therapeutic Peptide Review and Emerging Peptide Science

14:30 - 15:00
Peptide Discovery, Preclinical and Clinical

This session will provide an overview of the peptide therapeutic landscape, based on our comprehensive dataset of peptides that have entered human clinical studies. We will also highlight some emerging peptides from Ferring's discovery programs.

Participants

Michael Dunn - Senior Director, Ferring Research Institute

APL-2 Drug Substance: Development of a Commercial Generation 2 Process

14:30 - 15:00
Peptide Chemistry, Manufacturing and Controls

Participants

Najib Maslouh, PhD - Vice President of Manufacturing, Technical Operati, Apellis Pharmaceuticals

Inhibition and Degradation of Drug Targets Using bioPROTAC mRNAs – A Novel Approach with Broad Therapeutic Potential

14:30 - 15:00
mRNA Therapeutics and CRISPR Therapeutics

To tackle historically intractable targets, we are pursuing 'bioPROTACs', targeted-degradation fusion constructs composed of I) mini-proteins/peptides with high-affinity against therapeutic targets linked to II) truncated E3 ligase receptors. Provided that the mini-protein's binding affinity is below mid-nanomolar, its fold and target interaction site is flexible with efficient degradation achieved with monobodies, nanobodies, DARPins, αREPs and peptides. Similarly, there is flexibility for the truncated E3 protein with effective examples across several E3 classes. Coupled with mRNA delivery, bioPROTACs have several distinct advantages as a potential therapeutic modality including inhibit & degrade pharmacology, specificity against post-translation modifications, leveraging of disease-relevant E3s, employment of tissue-specific mRNA self-destruct sequences, and the potential to boost neoantigens. So far, the bioPROTAC approach has been broadly successful with active constructs against several targets with rapid and robust degradation activity across multiple cell lines. Currently, we are working towards in vivo delivery of bioPROTAC mRNAs via lipid nanoparticles. Prerequisite in vitro experiments with a bioPROTAC against proliferating cell nuclear antigen (PCNA) showed robust degradation and proliferation/apoptotic effects in a variety of cancer cell lines. Detailed studies in HepG2 cells showed degradation of PCNA, just 4 hours post-dosing and with just 100 pM mRNA.

Participants

Anthony Partridge, PhD - Principal Scientist, Early Discovery Pharmacology, Merck, Sharp & Dohme

Extra-hepatic Delivery

15:00 - 15:30
Oligonucleotide Discovery, Preclinical and Clinical

Alnylam Speaker TBA

Extra-hepatic Delivery

15:00 - 15:30
Oligonucleotide Chemistry, Manufacturing and Controls

Alnylam Speaker TBA

Biopharmaceutical Properties of Peptide:polyethylene Glycol Supramolecular Assemblies

15:00 - 15:30
Peptide Discovery, Preclinical and Clinical

We demonstrate non-covalent PEGylation of acylated therapeutic peptides as a strategy to circumvent potential loss of potency upon covalent conjugation, while maintaining a significant improvement in solubility, long term stability, and bioavailability following subcutaneous injection. The approach is amenable to both liquid and solid state peptide formulation strategies. In silico molecular modelling of the complexation between acylated peptide and PEG-cholane has directed analytical method development towards characterisation of physical quality attributes such as peptide:PEG molar stoichiometry, structure, aggregation and fibrillation.

Participants

Christopher van der Walle - Director, Fellow, Biopharmaceutical Development, MedImmune Ltd.

Development of Long Contiguous Overlapping Peptides for Ultra-Fast Allergy Immunotherapy

15:00 - 15:30
Peptide Chemistry, Manufacturing and Controls

Contiguous Overlapping Peptides (COPs) based vaccine provide a novel tool for allergen immunotherapy (AIT). COPs are long synthetic peptides, reproducing fragments of the amino sequence of a selected major allergen(s). The presentation reviews the production, analysis, characterization and formulation of Bet v1 COP, the major allergen in birch pollen.

Participants

Vanya Beltrami - VP, Head of Manufacturing, Anergis SA

TriMix Based mRNA Immunotherapies

15:00 - 15:30
mRNA Therapeutics and CRISPR Therapeutics

TriMix, a mixture of mRNAs that encodes CD40L, CD70 and caTLR4 has been specifically designed to enhance the interaction of DCs with T cells. In combination with mRNA encoding tumor-associated antigens (TAAs), TriMix acts as an adjuvant that enhances the TAA-specific T cell response. The magnitude and functional characteristics of T cell responses elicited by TriMix based mRNA vaccines are governed by the complex interplay between route of administration, the delivery vehicle applied and the intrinsic properties of the mRNA.

Participants

Stefaan De Koker, Ph.D - Non-clinical Principle Scientist and site-Director, eTheRNA

Targeted Delivery of Antisense Oligonucleotides to Extra-hepatic Tissues

15:30 - 16:00
Oligonucleotide Discovery, Preclinical and Clinical

Previous work targeting liver hepatocytes has shown large enhancement in potency and multiple GalNAc conjugated ASOs are making their way through clinical trials. To expand of this success, we have investigated various strategies to improve delivery and potency in extra-hepatic tissues. Herein will be discussed targeting approaches and mechanisms of ASO delivery to specific tissues.

Participants

Michael Oestergaard - Research Fellow, Ionis Pharmaceuticals

Targeted Delivery of Antisense Oligonucleotides to Extra-hepatic Tissues

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Participants

Michael Oestergaard - Research Fellow, Ionis Pharmaceuticals

SESSIONS

MAIN CONFERENCE DAY 2 - 14/11/2019

Engineered Amphiphilic Peptides Enable Delivery of Protein and CRISPR Cargoes to Cells

15:30 - 16:00
Peptide Discovery, Preclinical and Clinical

Among biologic cargoes, proteins offer promise but are limited by a lack of efficient delivery methods. We developed amphiphilic peptides that enable robust delivery of proteins to cells by a simple co-incubation. These carrier peptides are optimized to deliver peptides, antibodies and CRISPR ribonucleoprotein complex to cells, including hard-to-modify Natural Killer cells and airway epithelia.

Participants

David Guay, PhD - Research Director, Feldan Therapeutics

Peptide CMC Lessons Learned

15:30 - 16:00
Peptide Chemistry, Manufacturing and Controls

Speaker TBA

mRNA Therapeutic Development

15:30 - 16:00
mRNA Therapeutics and CRISPR Therapeutics

Participants

Dr. Amy Rabideau, Ph.D. - Senior Scientist, Moderna

Networking Refreshment Break in Poster and Exhibit Hall

16:00 - 16:30

Co-Chairpersons' Remarks

16:30 - 16:35
Oligonucleotide Discovery, Preclinical and Clinical

Participants

Muthiah (Mano) Manoharan, PhD - Senior Vice President of Drug Discovery, Alnylam Pharmaceuticals, Inc.

Dmitry Samarsky, PhD - Chief Technology Officer, Sirnaomics

Chairman's Remarks

16:30 - 16:35
Peptide Discovery, Preclinical and Clinical

Participants

Jurgen Machielse - Business Development Director Spherical Gels, Zeochem AG

Chairman's Remarks

16:30 - 16:35
Peptide Chemistry, Manufacturing and Controls

Participants

Jurgen Machielse - Business Development Director Spherical Gels, Zeochem AG

Chairperson's Remarks

16:30 - 16:35
mRNA Therapeutics and CRISPR Therapeutics

Strategies for the Delivery of Nucleic Acid Therapeutics

16:35 - 17:05
Oligonucleotide Discovery, Preclinical and Clinical

Efficient delivery of nucleic acid therapeutics is essential to afford potent, safe products. Delivery strategies differ depending on the nature of the nucleic acid payload and the intended therapeutic use. This presentation will review Genevants delivery platforms for oligonucleotides and mRNA in hepatic and extrahepatic applications.

Participants

Peter Lutwyche, PhD - Chief Technology Officer, Genevant Sciences Corporation

Complete Enzyme Catalysed Oligonucleotide Synthesis: From Single Nucleotides to Final Product

16:35 - 17:05
Oligonucleotide Chemistry, Manufacturing and Controls

In order to address the challenges of large scale oligonucleotide synthesis, namely scalability, sustainability and cost, we are developing an enzyme catalysed approach to oligonucleotide manufacture. Short oligonucleotides are synthesized from simple nucleotide based starting materials using enzymes by adding nucleotides sequentially. The short oligonucleotides are subsequently assembled in a single templated convergent step to generate the final product oligonucleotide. This assembly and product separation eliminates all chromatography from the process. All processes are run in aqueous solution improving both scalability and sustainability. The convergent approach improves overall process yields while the templating removes impurities such as 'N-1' sequences from the final product.

Participants

Martin Olbrich, Ph.D - Process Chemist, F Hoffmann-La Roche Ltd.

When Upstream Meets Downstream Processing: It Takes Two to Have an Efficient Manufacturing Process

16:35 - 17:00
Peptide Discovery, Preclinical and Clinical

Process development is often considered a stand-alone activity. This is not the case for most projects. In this presentation, Bachem will discuss process development in the context of new chemical entity projects from phase I up to commercialization of the drug substance.

Participants

Ralf Eisenhuth, PhD - Process Manager Technology Transfer and Chromatography, Bachem AG

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Peptide Chemistry, Manufacturing and Controls

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Participants

Ralf Eisenhuth, PhD - Process Manager Technology Transfer and Chromatography, Bachem AG

Messenger RNA Therapeutics for Primary Ciliary Dyskinesia

16:35 - 17:00
mRNA Therapeutics and CRISPR Therapeutics

Primary ciliary dyskinesa (PCD) is a genetically heterogenous hereditary disease syndrom originating from mutations in genes encoding proteins which are either essential for ciliogenesis or are structural/ functional parts of cilia. The dysfunction of motile cilia results in a reduced mucociliary clearance and consequently recurrent lung infections, which can lead to chronic destructive lung disease with bronchiectasis and progressive lung failure. Challenges in pulmonary delivery of mRNA will be discussed and proof of concept data for mRNA transcript therapy for PCD will be presented.

Participants

Christian Plank, PhD - Chief Technology Officer, Ethris GmbH

SESSIONS

MAIN CONFERENCE DAY 2 - 14/11/2019

Chemo-enzymatic Peptide Synthesis; Peptide Chain Length Becomes Less Relevant

17:00 - 17:30
Peptide Discovery, Preclinical and Clinical

The peptiligase technology platform has been successfully applied in the synthesis of medium sized peptide-based therapeutics like Exenatide, Thymosin-a1 and Liraglutide. It has been shown that synthesis yields could be improved by 20-30% compared to SPPS due to the enzymatic ligation strategy as well as analytical technology. This presentation will summarize the current status and will look forward to the possibilities around large(r) peptide constructs.

Participants

Leendert van ven Bos - Chief Executive Officer, EnzyTag BV

Chemo-enzymatic Peptide Synthesis; Peptide Chain Length Becomes Less Relevant

17:00 - 17:30
Peptide Chemistry, Manufacturing and Controls

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Participants

Leendert van ven Bos - Chief Executive Officer, EnzyTag BV

Self Amplifying mRNA (SAM) Vaccines for Rapid Response

17:00 - 17:30
mRNA Therapeutics and CRISPR Therapeutics

Based on our accumulated experience on alphavirus vectors, we developed a self-amplifying (SAM) mRNA vaccine. Here we show that delivery of a 9 kb self-amplifying RNA encapsulated within an LNP or adsorbed to CNE substantially increased potency compared to delivery of naked RNA, and had comparable or improved potency to more established vaccine approaches. This novel vaccine technology was tested with genes encoding antigens from several viral pathogens and found to elicit broad and potent immune responses.

Participants

Derek O'Hagan - Head of Global Discovery Support & New Technologie, GSK Vaccines

Developing an Engineered Exosome Therapeutics Platform

17:05 - 17:35
Oligonucleotide Discovery, Preclinical and Clinical

Exosomes have evolved to enable the transport of large and small macromolecules of various compositions across cellular barriers including the inner and outer cellular membranes. We have developed the engEx platform, our proprietary exosome engineering and manufacturing platform, to expand upon the innate properties of exosomes to design novel exosome therapeutics. Our engEx platform enables development of novel exosome product candidates that are uniquely designed to target cytoplasmic, nuclear or membrane signaling pathways throughout the body, with the goal of delivering potent signals to specific target cells. We will describe the development of initial engineered exosome therapeutic, exoSTING, designed to elicit potent anti-tumor immunity by selectively activating tumor resident antigen presenting cells.

Participants

Sriram Sathy - Vice President Biology and Translational Medicine, Codiak Biosciences

Process Improvement Strategies in Oligonucleotide Development and Manufacturing

17:05 - 17:35
Oligonucleotide Chemistry, Manufacturing and Controls

Nitto Avecia Speaker TBA

Cost Efficient Peptide & Oligonucleotide Purification via ZEOsphere DRP Mixed-Mode Chromatography

17:30 - 18:00
Peptide Discovery, Preclinical and Clinical

The workshop will show the beneficial use of ZEOsphere DRP Mixed-Mode stationary phases in the repulsive-attractive mode compared to RP or IEX stationary phases on crude peptides and oligonucleotides. ZEOsphere DRP orthogonal interaction is due to a better selectivity not only able to increase purity, recovery and loading, but also to decrease the organic solvent usage. Real Peptide and Oligonucleotide crude separation will be discussed.

Participants

Victoria Custodis - Team Leader R&D, Zeochem AG

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Participants

Victoria Custodis - Team Leader R&D, Zeochem AG

Semi-automated Manufacturing of mRNA Nanoparticle Products for Personalized Neoantigen-specific Cancer Immunotherapy

17:30 - 18:00
mRNA Therapeutics and CRISPR Therapeutics

Nanoparticle products comprising mRNA have been gaining attention for various therapeutic approaches including cancer immunotherapy, protein replacement, vaccination, and in vivo expression of therapeutic antibodies. For clinical development of such products, robust automated manufacturing processes and formulations suited for long-term stabilization of RNA nanoparticle are required. The presentation will deal with BioNtech's experiences during process and formulation development of a GMP compliant manufacturing process for a personalized clinical trial with a neoantigen-specific therapy using mRNA.

Participants

Sebastian Hörner - Head of Process Development, Formulation & Drug Development, BioNTech RNA Pharmaceuticals GmbH

TANGO (Targeted Augmentation of Nuclear Gene Output) for the Treatment of Genetic Diseases

17:35 - 18:05
Oligonucleotide Discovery, Preclinical and Clinical

TANGO (Targeted Augmentation of Nuclear Gene Output) is a novel technology which exploits antisense-mediated modulation of pre-mRNA splicing to increase protein expression. TANGO prevents naturally-occurring non-productive splicing events and increases the generation of productive mRNA, resulting in an increase of full-length, fully-functional protein. We are applying TANGO to develop treatments for autosomal dominant haploinsufficiencies.

Participants

Huw Nash, PhD - Chief Operating Officer and Chief Business Officer, Stoke Therapeutics

GalNAc Cluster: Process Chemistry and Regulatory Considerations

17:35 - 18:05
Oligonucleotide Chemistry, Manufacturing and Controls

Triantennary N-acetyl-D-galactosamine (GalNAc) clusters are well-established moieties for targeting oligonucleotide drugs to the liver by way of the asialoglycoprotein receptor (ASGPR). In this talk, the latest process development for the manufacturing process of the triantennary GalNAc cluster is presented. The knowledge generated on the origin and the fate of process impurities, along with the robust manufacturing process, provide key elements in support of the GalNAc cluster as proposed regulatory starting material.

Participants

David Tew - Senior Fellow, Glaxosmithkline Medical Reseach Centre

The Role of Membranes in (Pep)tide Synthesis: An Alternative Method of Addressing the Sustainability Challenges in Synthesis and Purification

18:00 - 18:30
Peptide Discovery, Preclinical and Clinical

This presentation will give an overview, illustrated by examples, of how membranes, and in particular membranes stable to organic solvents, can be beneficial in peptide and oligonucleotide synthesis. Examples will range from solvent exchange, to purification and reaction integrated membrane processes. Particular attention will be given to new membrane developments being carried out within Vito and how these can aid large scale production of tides.

Participants

Dominic Ormerod, Ph.D - Project Manager, Process Intensification, VITO

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Participants

Dominic Ormerod, Ph.D - Project Manager, Process Intensification, VITO

Regulatory View on mRNA Challenges in the Context of ATMP/Gene Therapy Guidelines

18:00 - 18:30
mRNA Therapeutics and CRISPR Therapeutics

There are specific guidance's for ATMP/Gene Therapy from EU CHMP and US FDA available. The challenge is that mRNA constructs are not classical Gene Therapeutics and therefore the full applicability of these guidelines could be questioned. The presentation tries to outline pitfalls, challenges and opportunities for the CMC, nonclinical and clinical areas by focusing on the most relevant guidances.

Participants

Otmar Pfaff - Vice President, Regulatory Affairs, CureVac AG

MicroRNAs - An Emerging Attractive Drug Discovery Platform for Therapeutic Intervention in Oncology

18:05 - 18:35
Oligonucleotide Discovery, Preclinical and Clinical

MicroRNAs represent a class of small (18- to 28-nt), naturally-occuring non-coding RNA molecules which play major roles in normal and in pathological cellular processes, such as cell differentiation, cell cycle progression, and apoptosis. Unlike antisense oligonucleotides or siRNAs, microRNAs are able to target multiple genes (100+) simultaneously, thus modulating the expression of numerous proteins in key biological pathways/networks.

Participants

Dr. Michel Janicot, Ph.D - Chief Development Officer, InteRNA Technologies

Late Breaking Presentation

18:05 - 18:35
Oligonucleotide Chemistry, Manufacturing and Controls

Close of Day

18:35 - 18:40

SCHEDULE

MAIN CONFERENCE DAY 2 - 14/11/2019

TIME	KEYNOTE/PLENARY SESSION	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	PEPTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY, PRECLINICAL AND CLINICAL	REGULATORY STRATEGIES FOR OLIGONUCLEOTIDES AND PEPTIDES	SPONSORED SPOTLIGHT PRESENTATION 1	SPONSORED SPOTLIGHT PRESENTATION 2	SPONSORED SPOTLIGHT PRESENTATION 3	SPONSORED SPOTLIGHT PRESENTATION 4	MRNA THERAPEUTICS AND CRISPR THERAPEUTICS
08:00	**08:00** - Sponsored Breakfast Spotlight Presentation **08:40** - Chairperson's Remarks **08:45** - Global Development Programme of RG6042, an Antisense Oligonucleotide, for the Treatment of Huntington's Disease	**08:00** - Sponsored Breakfast Spotlight Presentation	**08:00** - Sponsored Breakfast Spotlight Presentation	**08:00** - Sponsored Breakfast Spotlight Presentation	**08:00** - Sponsored Breakfast Spotlight Presentation	**08:00** - Sponsored Breakfast Spotlight Presentation	**08:00** - Sponsored Breakfast Spotlight Presentation	**08:00** - Sponsored Breakfast Spotlight Presentation	**08:00** - Sponsored Breakfast Spotlight Presentation	**08:00** - Sponsored Breakfast Spotlight Presentation	**08:00** - Sponsored Breakfast Spotlight Presentation

SCHEDULE

MAIN CONFERENCE DAY 2 - 14/11/2019

TIME	KEYNOTE/PLENARY SESSION	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	PEPTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY, PRECLINICAL AND CLINICAL	REGULATORY STRATEGIES FOR OLIGONUCLEOTIDES AND PEPTIDES	SPONSORED SPOTLIGHT PRESENTATION 1	SPONSORED SPOTLIGHT PRESENTATION 2	SPONSORED SPOTLIGHT PRESENTATION 3	SPONSORED SPOTLIGHT PRESENTATION 4	MRNA THERAPEUTICS AND CRISPR THERAPEUTICS
09:00	**09:15** - Plants as Biofactories for Producing Peptide-based Pharmaceuticals **09:45** - Regulatory Quality and CMC Perspectives on mRNA Vaccines and Peptide Vaccine Adjuvants										
10:00	**10:15** - Networking Refreshment Break in Poster and Exhibit Hall	**10:15** - Networking Refreshment Break in Poster and Exhibit Hall	**10:15** - Networking Refreshment Break in Poster and Exhibit Hall	**10:15** - Networking Refreshment Break in Poster and Exhibit Hall	**10:15** - Networking Refreshment Break in Poster and Exhibit Hall	**10:15** - Networking Refreshment Break in Poster and Exhibit Hall **10:55** - A Case Study of Concurrent Global Regulatory Filings for Two Oligonucleotides	**10:15** - Networking Refreshment Break in Poster and Exhibit Hall	**10:15** - Networking Refreshment Break in Poster and Exhibit Hall	**10:15** - Networking Refreshment Break in Poster and Exhibit Hall	**10:15** - Networking Refreshment Break in Poster and Exhibit Hall	**10:15** - Networking Refreshment Break in Poster and Exhibit Hall

SCHEDULE

MAIN CONFERENCE DAY 2 - 14/11/2019

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11:00						**11:25** - Experience with Early and Late Phase Global Submissions of Oligonucleotide-based Products **11:55** - Peptide Regulatory Strategies and Experiences					
12:00						**12:25** - Transition to Spotlight Presentation Rooms	**12:30** - Mass-Production of Target RNA by Microorganism	**12:30** - Quantification of Selected Impurities in Oligonucleotides	**12:30** - Precision Nanosystems Briefing	**12:30** - Changing the Tide in Peptides - Peptide Purification and Modification with Beylntic's Catch-and-Release Technology	
13:00	**13:00** - Networking Luncheon in Poster and Exhibit Hall	**13:00** - Networking Luncheon in Poster and Exhibit Hall	**13:00** - Networking Luncheon in Poster and Exhibit Hall	**13:00** - Networking Luncheon in Poster and Exhibit Hall	**13:00** - Networking Luncheon in Poster and Exhibit Hall	**13:00** - Networking Luncheon in Poster and Exhibit Hall	**13:00** - Networking Luncheon in Poster and Exhibit Hall	**13:00** - Networking Luncheon in Poster and Exhibit Hall	**13:00** - Networking Luncheon in Poster and Exhibit Hall	**13:00** - Networking Luncheon in Poster and Exhibit Hall	**13:00** - Networking Luncheon in Poster and Exhibit Hall

SCHEDULE

MAIN CONFERENCE DAY 2 - 14/11/2019

TIME	KEYNOTE/PLENARY SESSION	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	PEPTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY, PRECLINICAL AND CLINICAL	REGULATORY STRATEGIES FOR OLIGONUCLEOTIDES AND PEPTIDES	SPONSORED SPOTLIGHT PRESENTATION 1	SPONSORED SPOTLIGHT PRESENTATION 2	SPONSORED SPOTLIGHT PRESENTATION 3	SPONSORED SPOTLIGHT PRESENTATION 4	MRNA THERAPEUTICS AND CRISPR THERAPEUTICS
14:00		**14:25** - Co-Chairpersons' Remarks **14:30** - Overcoming Extra- and Intracellular Barriers: Polymer-based mRNA Delivery Systems	**14:25** - Co-Chairpersons' Remarks **14:30** - Overcoming Extra- and Intracellular Barriers: Polymer-based mRNA Delivery Systems	**14:25** - Chairperson's Remarks **14:30** - APL-2 Drug Substance: Development of a Commercial Generation 2 Process	**14:25** - Chairperson's Remarks **14:30** - Therapeutic Peptide Review and Emerging Peptide Science						**14:25** - Chairperson's Remarks **14:30** - Inhibition and Degradation of Drug Targets Using bioPROTAC mRNAs – A Novel Approach with Broad Therapeutic Potential

SCHEDULE

MAIN CONFERENCE DAY 2 - 14/11/2019

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15:00		**15:00** - Extrahepatic Delivery **15:30** - Targeted Delivery of Antisense Oligonucleotides to Extra-hepatic Tissues	**15:00** - Extrahepatic Delivery **15:30** - Targeted Delivery of Antisense Oligonucleotides to Extra-hepatic Tissues	**15:00** - Development of Long Contiguous Overlapping Peptides for Ultra-Fast Allergy Immunotherapy **15:30** - Peptide CMC Lessons Learned	**15:00** - Biopharmaceutical Properties of Peptide:polyethylene Glycol Supramolecular Assemblies **15:30** - Engineered Amphiphilic Peptides Enable Delivery of Protein and CRISPR Cargoes to Cells						**15:00** - TriMix Based mRNA Immunotherapies **15:30** - mRNA Therapeutic Development

SCHEDULE

MAIN CONFERENCE DAY 2 - 14/11/2019

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16:00	**16:00** - Networking Refreshment Break in Poster and Exhibit Hall	**16:00** - Networking Refreshment Break in Poster and Exhibit Hall **16:35** - Complete Enzyme Catalysed Oligonucleotide Synthesis: From Single Nucleotides to Final Product	**16:00** - Networking Refreshment Break in Poster and Exhibit Hall **16:30** - Co-Chairpersons' Remarks **16:35** - Strategies for the Delivery of Nucleic Acid Therapeutics	**16:00** - Networking Refreshment Break in Poster and Exhibit Hall **16:30** - Chairman's Remarks **16:35** - When Upstream Meets Downstream Processing: It Takes Two to Have an Efficient Manufacturing Process	**16:00** - Networking Refreshment Break in Poster and Exhibit Hall **16:30** - Chairman's Remarks **16:35** - When Upstream Meets Downstream Processing: It Takes Two to Have an Efficient Manufacturing Process	**16:00** - Networking Refreshment Break in Poster and Exhibit Hall	**16:00** - Networking Refreshment Break in Poster and Exhibit Hall	**16:00** - Networking Refreshment Break in Poster and Exhibit Hall	**16:00** - Networking Refreshment Break in Poster and Exhibit Hall	**16:00** - Networking Refreshment Break in Poster and Exhibit Hall	**16:00** - Networking Refreshment Break in Poster and Exhibit Hall **16:30** - Chairperson's Remarks **16:35** - Messenger RNA Therapeutics for Primary Ciliary Dyskinesia

SCHEDULE

MAIN CONFERENCE DAY 2 - 14/11/2019

TIME	KEYNOTE/PLENARY SESSION	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	PEPTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY, PRECLINICAL AND CLINICAL	REGULATORY STRATEGIES FOR OLIGONUCLEOTIDES AND PEPTIDES	SPONSORED SPOTLIGHT PRESENTATION 1	SPONSORED SPOTLIGHT PRESENTATION 2	SPONSORED SPOTLIGHT PRESENTATION 3	SPONSORED SPOTLIGHT PRESENTATION 4	MRNA THERAPEUTICS AND CRISPR THERAPEUTICS
17:00		**17:05** - Process Improvement Strategies in Oligonucleotide Development and Manufacturing **17:35** - GalNAc Cluster: Process Chemistry and Regulatory Considerations	**17:05** - Developing an Engineered Exosome Therapeutics Platform **17:35** - TANGO (Targeted Augmentation of Nuclear Gene Output) for the Treatment of Genetic Diseases	**17:00** - Chemoenzymatic Peptide Synthesis; Peptide Chain Length Becomes Less Relevant **17:30** - Cost Efficient Peptide & Oligonucleotide Purification via ZEOsphere DRP Mixed-Mode Chromatography	**17:00** - Chemoenzymatic Peptide Synthesis; Peptide Chain Length Becomes Less Relevant **17:30** - Cost Efficient Peptide & Oligonucleotide Purification via ZEOsphere DRP Mixed-Mode Chromatography						**17:00** - Self Amplifying mRNA (SAM) Vaccines for Rapid Response **17:30** - Semi-automated Manufacturing of mRNA Nanoparticle Products for Personalized Neoantigen-specific Cancer Immunotherapy

SCHEDULE

MAIN CONFERENCE DAY 2 - 14/11/2019

TIME	KEYNOTE/PLENARY SESSION	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	PEPTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY, PRECLINICAL AND CLINICAL	REGULATORY STRATEGIES FOR OLIGONUCLEOTIDES AND PEPTIDES	SPONSORED SPOTLIGHT PRESENTATION 1	SPONSORED SPOTLIGHT PRESENTATION 2	SPONSORED SPOTLIGHT PRESENTATION 3	SPONSORED SPOTLIGHT PRESENTATION 4	MRNA THERAPEUTICS AND CRISPR THERAPEUTICS
18:00	**18:35** - Close of Day	**18:05** - Late Breaking Presentation **18:35** - Close of Day	**18:05** - MicroRNAs - An Emerging Attractive Drug Discovery Platform for Therapeutic Intervention in Oncology **18:35** - Close of Day	**18:00** - The Role of Membranes in (Pep)tide Synthesis: An Alternative Method of Addressing the Sustainability Challenges in Synthesis and Purification **18:35** - Close of Day	**18:00** - The Role of Membranes in (Pep)tide Synthesis: An Alternative Method of Addressing the Sustainability Challenges in Synthesis and Purification **18:35** - Close of Day	**18:35** - Close of Day	**18:35** - Close of Day	**18:35** - Close of Day	**18:35** - Close of Day	**18:35** - Close of Day	**18:00** - Regulatory View on mRNA Challenges in the Context of ATMP/Gene Therapy Guidelines **18:35** - Close of Day

SESSIONS

MAIN CONFERENCE DAY 3 - 15/11/2019

Sponsored Breakfast Spotlight Presentation

08:15 - 08:45

Chairman's Remarks

08:55 - 09:00
Oligonucleotide Discovery, Preclinical and Clinical

Chairman's Remarks

08:55 - 09:00
Oligonucleotide Chemistry, Manufacturing and Controls

Participants

Yogesh Sanghvi, PhD - President, Rasayan Inc.

Chairman's Remarks

08:55 - 09:00
Peptide Discovery, Preclinical and Clinical

Chairman's Remarks

08:55 - 09:00
Peptide Chemistry, Manufacturing and Controls

Chairman's Remarks

08:55 - 09:00
mRNA Therapeutics and CRISPR Therapeutics

ApTOLL, A New Therapeutic Approach for the Treatment of Ischemic Stroke

09:00 - 09:30
Oligonucleotide Discovery, Preclinical and Clinical

ApTOLL is an aptamer designed to block TLR4, a crucial receptor involved in the inflammatory response triggered after ischemic stroke. Efficacy of ApTOLL has been demonstrated in experimental models of stroke and safety has been assessed in regulatory assays. Phase I in healthy volunteers currently ongoing.

Participants

Macarena Hernández-Jiménez - Chief Scientific Officer, AptaTargets S.L.

Considerations for Ton-scale Oligonucleotide Manufacturing via Solid-phase synthesis, Preparing for the Future

09:00 - 09:30
Oligonucleotide Chemistry, Manufacturing and Controls

Although there are new methodologies for oligonucleotide synthesis on the horizon (e.g. solution-phase synthesis), the tried and true approach for the past 20 years has been a solid-phase route. In this approach, what will ton-scale production look like? What are likely failure modes and considerations not immediately visible in current kilogram-scale applications.

Participants

Isaiah Cedillo - Director, Manufacturing & Operations, Ionis Pharmaceuticals

Development of Personal Neoantigen Cancer Vaccine NEO-PV-01

09:00 - 09:30
Peptide Discovery, Preclinical and Clinical

Neon Therapeutics, a clinical-stage immuno-oncology company developing neoantigen-based therapeutics, has pioneered a proprietary neoantigen platform to develop a personal cancer vaccine, NEO-PV-01. The neoantigen-targeting peptides in the vaccine are intended to engage the immune system to precisely and selectively attack tumors. Our objective is to create and deepen anti-tumor immune responses and broaden the range of cancers treatable via immuno-oncology approaches. Immune and clinical data from our first clinical trial NT-001 will be summarized. In addition, the manufacturing process for NEO-PV-01 will be discussed including the automated and scalable peptide production that we believe provide advantages in both turnaround times and manufacturing capacities.

Participants

Jesse Dong, PhD - Vice President, Peptide Chemistry, Neon Therapeutics

Development of Personal Neoantigen Cancer Vaccine NEO-PV-01

09:00 - 09:30
Peptide Chemistry, Manufacturing and Controls

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Participants

Jesse Dong, PhD - Vice President, Peptide Chemistry, Neon Therapeutics

SESSIONS

MAIN CONFERENCE DAY 3 - 15/11/2019

SiRNA therapeutics for Oncology: New Avenues to Success

09:30 - 10:00
Oligonucleotide Discovery, Preclinical and Clinical

Immune Checkpoint (IC) inhibitors for Hepatocellular Carcinoma (HCC) have only a low level of success since an increase in TGFbeta around the tumor prevents T-cell penetration and activation. Silencing TGFbeta near the tumor demonstrated single agent activity in HCC. In the presence of an IC antibody (anti-PDL1), it resulted in increased T-cell penetration deeper into the tumor. This presentation will outline the benefits of Sirnaomics vehicles for multiple siRNA delivery and the steps being taken to drive these innovations to the clinic as cancer therapeutics.

Participants

David Evans, PhD - Co-Founder and CSO, Sirnaomics

Presentation Title TBA

09:30 - 10:00
Oligonucleotide Chemistry, Manufacturing and Controls

Participants

Carl 'Charlie' Hitscherich - Global Head of Clinical Supply Chain, Alnylam Pharmaceuticals

Individualized Neoantigen-specific Therapy against Cancer Using messenger RNA

09:30 - 10:00
Peptide Discovery, Preclinical and Clinical

One of the obviously biggest challenges in fighting cancer is that every patient has a unique cancer. One reasonable approach to overcome this is to target the unique molecular signature of each cancer and to adapt treatment to the patient's disease and immune system dynamics. To deliver the genetic information of antigens into antigen-presenting dendritic cells of the immune system the mRNA platform technology offers plenty of new options. The presentation will deal with conducting a clinical trial with a neoantigen-specific therapy using mRNA.

Participants

Dr. Christoph Kroener - Head of IVAC Mutanome Lead Structure, BioNTech AG

Individualized Neoantigen-specific Therapy against Cancer Using messenger RNA

09:30 - 10:00
Peptide Chemistry, Manufacturing and Controls

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Individualized Neoantigen-specific Therapy against Cancer Using messenger RNA

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Participants

Dr. Christoph Kroener - Head of IVAC Mutanome Lead Structure, BioNTech AG

Inhibition of microRNA-155 as a Therapeutic Strategy for the Treatment of Hematological Malignancies

10:00 - 10:30
Oligonucleotide Discovery, Preclinical and Clinical

microRNA-155 regulates the expression of multiple pathways implicated in oncology and its overexpression is an indicator of poor prognosis in several hematological malignancies and solid tumors. Clinical studies of cobomarsen, an inhibitor of microRNA-155, in cutaneous T-cell lymphoma and adult T-cell leukemia/lymphoma suggest a potential role for cobomarsen in the treatment miR-155 elevated hematological malignancies.

Participants

William Marshall, PhD - President and Chief Executive Officer, miRagen Therapeutics, Inc.

New Approaches in Oligonucleotide Manufacturing

10:00 - 10:30
Oligonucleotide Chemistry, Manufacturing and Controls

Manufacturing large quantities of oligonucleotides for therapeutic uses is a challenge. The batch mode solid-phase synthesis at a few kilogram scale is very efficient. Scale-out of the process is technically feasible, but challenging economically, because requires expensive equipment and facility that can accommodate the large volume of solvents and waste. In this presentation, our efforts to solve some of the issues for large-scale manufacturing will be discussed.

Participants

Xianglin Shi - Principal Scientist, Biogen

Delivering of Novel Cure: Unbridling the Power of mRNA-based Medicines

10:00 - 10:30
Peptide Discovery, Preclinical and Clinical

As the first biotech company in China to focus on mRNA therapeutics, Stemirna strives to deliver novel cure for patients in the world. Taking advantage of its two innovative mRNA platforms – a comprehensive mRNA delivery platform and an IVT-mRNA synthesis platform – Stemirna Therapeutics turns the patient's body into a drug factory that produces medicines for itself. Stemirna's current pipeline include personalized cancer vaccines and prophylactic vaccines for infectious diseases.

Participants

Dr. Hangwen Li - Chairman and CEO, Stemirna Therapeutics

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Participants

Dr. Hangwen Li - Chairman and CEO, Stemirna Therapeutics

Delivering of Novel Cure: Unbridling the Power of mRNA-based Medicines

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mRNA Therapeutics and CRISPR Therapeutics

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Participants

Dr. Hangwen Li - Chairman and CEO, Stemirna Therapeutics

Networking Refreshment Break

10:30 - 11:00

Clinical Development of CXCL12 Inhibiting L-RNA Aptamer NOX-A12 (Olaptesed Pegol)

11:00 - 11:30
Oligonucleotide Discovery, Preclinical and Clinical

The CXCL12-neutralizing, PEGylated L-RNA aptamer (Spiegelmer®) NOX-A12 was tested as monotherapy and in combination with the PD-1 checkpoint inhibitor pembrolizumab in a Phase 1/2 study in metastatic microsatellite-stable colorectal and pancreatic cancer. Pharmacodynamic effects on the tumor microenvironment in response to the NOX-A12 monotherapy and efficacy and safety data for the combination with pembrolizumab as well as future plans in these and additional indications will be presented.

Participants

Dirk Eulberg - Vice President Project Management, NOXXON Pharma AG

Trinucleotide Phosphoramidites: Synthons for Codon-based Gene Synthesis and Blockmers for Oligonucleotide Assembly

11:00 - 11:30
Oligonucleotide Chemistry, Manufacturing and Controls

Trinucleotide synthons stand out as facilitating fully controlled randomization at any number and position of codons of a given gene. We have been working on protocols that give easy access to such trinucleotides (blockmers), which are also promising synthons for the general assembly of oligonucleotides.

Participants

Sabine Muller - Professor, University of Greifswald

Applying New Imaging Modalities to the ADME of ASO and Peptide Drugs

11:00 - 11:30
Peptide Discovery, Preclinical and Clinical

The ability to detect and quantify therapeutic ASOs and peptides is pivotal to their development as viable drug candidates, especially when characterizing novel delivery agents. Emerging tissue MS and non-invasive optical methodologies are providing new opportunities to accurately determine the ADME and efficacy of these macromolecular medicines.

Participants

Steve Hood - Director, Bioimaging and D@T, GlaxoSmithKline

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mRNA Therapeutics and CRISPR Therapeutics

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Participants

Steve Hood - Director, Bioimaging and D@T, GlaxoSmithKline

The Oligonucleotide Agent BC 007 for Neutralization of Pathogenic Agonistic Autoantibodies Directed Against ß 1-Adrenoceptors in Heart Failure Patients – Up-date with Very First Phase II Data

11:30 - 12:00
Oligonucleotide Discovery, Preclinical and Clinical

BC 007, a 15mer ssDNA sequence consisting of nine guanosine and six thymidine nucleosides neutralizes functional active pathogenic autoantibodies against the ß 1-adrenoceptor (ß 1-AAb) that are cause of heart failure (HF) with high prevalence. Other autoantibodies of this class against G-protein-coupled receptors (GPCR-AAb) and being associated with different diseases are also neutralized as already shown in GPCR-AAb positive elderly healthy volunteers (Phase-1, Part C). Phase-1 safety tests showed an excellent tolerability, no clinically relevant side effects. Transient elevated aPTT to subclinical values, paralleling the infusion, were observed in some subjects. BC 007 is rapidly metabolized unexpectedly even down to its nucleic bases degradation products beta-aminoisobutyric acid and uric acid, beginning shortly after start of infusion. BC 007 is now tested as the first causative drug for patients with ß 1-AAb associated heart failure. A two-arm randomized, open-label run-in phase (IIa) is currently investigating the temporal persistence of the ß 1-AAb neutralization, safety and PK in chronic HF patients with reduced ejection fraction, including twenty ß 1-AAb positive HF patients and ten positive patients served as controls. Here the very first outcome data will be presented.

Participants

Dr. Johannes Müller - CEO and Founder, Berlin Cures

SESSIONS

MAIN CONFERENCE DAY 3 - 15/11/2019

Nanostar Sieving for Liquid Phase Synthesis of Oligonucleotides

11:30 - 12:00
Oligonucleotide Chemistry, Manufacturing and Controls

Solid phase synthesis offers rapid synthesis of oligos and is the dominant technology at present. The attachment of the growing oligo to the solid phase enables effective separation of molecular debris during the coupling cycle. However, it is difficult to monitor the reaction progress, mass transfer resistances inside the resin can make achieving complete reactions difficult, and when it's time to increase capacity, numbering out of synthesisers or scale up of packed beds is required. This talk will present Nanostar Sieving, an alternative approach in which two or more growing oligos are linked to a hub molecule, forming a high molecular weight, macro-molecular nanostar. This nanostar is soluble, and is readily separated from molecular debris using a solvent stable nanofiltration membrane. The coupling cycles are carried out entirely in the liquid phase, with diafiltration to remove debris at each coupling and deprotection step. This allows further building block or other reagents to be added at each cycle, if any incomplete reaction is detected, and generally promotes a high degree of in-line quality control using uPLC-MS and other analytical techniques. The synthesis of a 2'-methyl RNA phosphorothioate 20 mer sequence using this approach at the 10-20 mmol (50-100 g) scale will be discussed, and the high purity of the crude material produced by Nanostar Sieving compared to the purity obtained by solid phase synthesis with similar excesses of phosphoramidites. Scale-up from the 1-2 g scale to the 50-100g scale has been achieved with spiral wound membrane modules, and will be described with an approximate economic comparison made with solid phase synthesis.

Participants

Andrew Livingston - Professor of Chemical Engineering, Imperial College London

Targeted Drug Delivery to the CNS and Peripheral Tissues Using the VECTrans® Innovative Technology

11:30 - 12:00
Peptide Discovery, Preclinical and Clinical

Initially designed to enhance the transport across the Blood-Brain Barrier (BBB), the VECTrans® technology developed by VECT-HORUS promotes the delivery of drugs through vectors targeting specific receptors expressed at the BBB or in specific peripheral organs or tumours. The technology will be exemplified with small organic or large protein payloads.

Participants

Dr. Guillaume Jacquot, Ph.D - Translational Research Manager, Vect-Horus

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Transition to Spotlight Presentation Rooms

12:00 - 12:05

Sponsored Spotlight Presentations

12:05 - 12:35

Networking Luncheon

12:35 - 13:40

Chairperson's Remarks

13:40 - 13:45
Oligonucleotide Discovery, Preclinical and Clinical

Chairperson's Remarks

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Oligonucleotide Chemistry, Manufacturing and Controls

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mRNA Therapeutics and CRISPR Therapeutics

Clinical Development of Tivanisiran, A siRNA for the Treatment of Dry Eye Disease

13:45 - 14:15
Oligonucleotide Discovery, Preclinical and Clinical

Tivanisiran is a siRNA inhibitor of the Transient Receptor Potential cation channel subfamily V member 1 (TRPV1) synthesis designed to reduce signs and symptoms of dry eye disease. Previous phase 2 trials identified the most effective dose of tivanisiran (1.125%) that has been tested in new phase 3 study. Sylentis will present topline results of phase 3 trial with updates on the clinical development of tivanisiran.

Participants

Anne-Marie Bleau - Clinical Operations Manager, Sylentis

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SESSIONS

MAIN CONFERENCE DAY 3 - 15/11/2019

Control Strategies and Analytical Test Methods for Peptide-Conjugates

13:45 - 14:15
Peptide Discovery, Preclinical and Clinical

Peptide conjugates constitute a promising and growing class of peptide therapeutics. They require a specific set of analytical test methods to fully characterize the manufacturing process, its intermediates and the final drug substance. Typically, the physical and chemical properties of the drug substances are substantially influenced or dominated by the properties of the conjugate. E.g. when a protein is conjugated to a peptide, biomolecular test methods are best suited, while PEGylated peptides require analytical test methods that adequately characterize the polymeric moiety. In addition, the determination of the degree of conjugation and the identification and structural elucidation of peptide related impurities in the drug substance are important for accurate characterization. We will present data from case studies, and discuss challenges during development of assay and purity methods while applying different chromatographic and MS techniques.

Participants

Silvan Rihm - Group Leader QC, Bachem AG

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Participants

Silvan Rihm - Group Leader QC, Bachem AG

Strategies for Translocation Reduction during CRISPR/Cas Multiplexing

13:45 - 14:15
mRNA Therapeutics and CRISPR Therapeutics

We have developed and characterized multiple strategies with which to reduce rearrangement frequencies. We observed that multi-gene editing with a CRISPR-Cas9 and CRISPR-Cas12a (Cpf1) combination reduced translocation frequencies compared to multiplexing with only CRISPR-Cas9. Taken together, for the development of T cell-based medicines, these data suggest that CRISPR-Cas12a is both robust, specific, and capable of reducing genomic rearrangements when making multiple gene edits compared to the CRISPR-Cas9 system alone.

Participants

John Zuris - Scientist III, Editas Medicine

Breakthrough Treatment with Twice a Year Shots to Prevent Heart Attacks and Strokes: Inclisiran Is a New Class of Cholesterol Lowering Drugs

14:15 - 14:45
Oligonucleotide Discovery, Preclinical and Clinical

Cardiovascular disease is the world's leading killer. Treatments with statins and PCSK9 mAbs are constrained by poor adherence. Inclisiran is in Phase III trials of twice-annual injections to lower LDL-C. If approved, inclisiran may help in millions of patients. We review challenges and progress, manufacturing tons of this siRNA.

Participants

John Richards - SVP and Head of Pharmaceutical Development, The Medicines Company

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John Richards - SVP and Head of Pharmaceutical Development, The Medicines Company

From Homebrewing to Peptide Chemistry

14:15 - 14:45
Peptide Discovery, Preclinical and Clinical

In this lecture, we would like to share with the peptide community our efforts in the quest of efficient methods and tools for the large-scale production of peptides as drugs. The tool that we are going to disclose is not used currently in the manufacture of peptides and we are convinced that it will be inspiring for any peptide chemist.

Participants

John Lopez - Fellow, Chemical R&D, Novartis AG

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Participants

John Lopez - Fellow, Chemical R&D, Novartis AG

Highly Efficient Gene Knockdown of Mouse Liver Genes Via Base Editing Using Non-viral Delivery of CRISPR-Cas9 Base Editors

14:15 - 14:45
mRNA Therapeutics and CRISPR Therapeutics

Base editing is a new category of genome editing. It enables precise, programmable conversion of single nucleotides in the mammalian genome without cutting DNA. Using a lipid nanoparticle-mediated delivery system, we report highly efficient disruption of mouse liver genes via introduction of a stop codon through precise base editing.

Participants

Dr. Francine Gregoire - VP, Liver Diseases, Beam Therapeutics

Givosiran Phase 3 Data and Next Steps

14:45 - 14:50
Oligonucleotide Discovery, Preclinical and Clinical

Alnylam Clinical Speaker TBA

Givosiran Phase 3 Data and Next Steps

14:45 - 14:50
Oligonucleotide Chemistry, Manufacturing and Controls

Alnylam Clinical Speaker TBA

Late Breaking Presentation

14:45 - 15:15
Peptide Discovery, Preclinical and Clinical

Late Breaking Presentation

14:45 - 15:15
Peptide Chemistry, Manufacturing and Controls

Investigation of Lipid Nanoparticle Formulation Optimisations across Nucleic acid-based Modalities

14:45 - 15:15
mRNA Therapeutics and CRISPR Therapeutics

Lipid nanoparticles are the most advanced clinically developed delivery system for nucleic acids. Many of these systems were first developed for siRNA, however in recent times there has been increasing interest in other nucleic acid-based modalities, in particular, mRNA and CRISPR-based gene editing which can be delivered via a DNA, mRNA/gRNA complex or ribonucleoprotein/gRNA complex. These modalities have a different mode of action and physicochemical properties. Here we present a series of formulation process and composition optimisation of LNP based systems and compare how they perform across different modalities. We find that LNP based systems effectively delivery a range of different physicochemical distinct cargo, highlighting the versatility of this platform as an intracellular delivery system. We also see specific optimisations are required to optimise depending on the cargo, providing insights into how to best optimise LNP based systems for a specific application.

Participants

Lili Cui - Senior Formulation Scientist, Pharm Sci, Astrazeneca

Close of TIDES Europe 2019

14:50 - 14:55
Oligonucleotide Discovery, Preclinical and Clinical

Close of TIDES Europe 2019

14:50 - 14:55
Oligonucleotide Chemistry, Manufacturing and Controls

Close of TIDES Europe 2019

15:15 - 15:20
Peptide Discovery, Preclinical and Clinical

Close of TIDES Europe 2019

15:15 - 15:20
Peptide Chemistry, Manufacturing and Controls

Nanocarrier for CRISPR Gene Editing and mRNA-mediated Tumor Suppressor Rescue

15:15 - 15:45
mRNA Therapeutics and CRISPR Therapeutics

DivInCell has designed a peptide-based nanocarrier that can potently deliver active CRISPR/CAS and mRNA in primary cell lines and in various tissues in vivo. We demonstrated that our proprietary nanocarrier promotes in vivo delivery CRISPR/Cas, leading to a robust editing of a selected target gene in specific organs or in tumors. This delivery technology was successfully applied in clinically relevant systems for CRISPR PCSK9 gene editing as well as for mRNA-mediated tumor suppressor rescue in pancreatic and ovarian cancers.

Gilles Divita, Ph.D., Chief Executive Officer, Divincell SAS, France

Participants

Gilles Divita - CEO, Divincell SAS

Close of TIDES Europe 2019

15:45 - 15:50
mRNA Therapeutics and CRISPR Therapeutics

SCHEDULE

MAIN CONFERENCE DAY 3 - 15/11/2019

TIDES Europe: Oligonucleotide and Peptide Therapeutics

12-15 November 2019
RAI Amsterdam
Amsterdam, Netherlands

TIME	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	PEPTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY, PRECLINICAL AND CLINICAL	MRNA THERAPEUTICS AND CRISPR THERAPEUTICS
08:00	**08:15** - Sponsored Breakfast Spotlight Presentation **08:55** - Chairman's Remarks	**08:15** - Sponsored Breakfast Spotlight Presentation **08:55** - Chairman's Remarks	**08:15** - Sponsored Breakfast Spotlight Presentation **08:55** - Chairman's Remarks	**08:15** - Sponsored Breakfast Spotlight Presentation **08:55** - Chairman's Remarks	**08:15** - Sponsored Breakfast Spotlight Presentation **08:55** - Chairman's Remarks
09:00	**09:00** - Considerations for Ton-scale Oligonucleotide Manufacturing via Solid-phase synthesis, Preparing for the Future **09:30** - Presentation Title TBA	**09:00** - ApTOLL, A New Therapeutic Approach for the Treatment of Ischemic Stroke **09:30** - SiRNA therapeutics for Oncology: New Avenues to Success	**09:00** - Development of Personal Neoantigen Cancer Vaccine NEO-PV-01 **09:30** - Individualized Neoantigen-specific Therapy against Cancer Using messenger RNA	**09:00** - Development of Personal Neoantigen Cancer Vaccine NEO-PV-01 **09:30** - Individualized Neoantigen-specific Therapy against Cancer Using messenger RNA	**09:00** - Development of Personal Neoantigen Cancer Vaccine NEO-PV-01 **09:30** - Individualized Neoantigen-specific Therapy against Cancer Using messenger RNA
10:00	**10:00** - New Approaches in Oligonucleotide Manufacturing **10:30** - Networking Refreshment Break	**10:00** - Inhibition of microRNA-155 as a Therapeutic Strategy for the Treatment of Hematological Malignancies **10:30** - Networking Refreshment Break	**10:00** - Delivering of Novel Cure: Unbridling the Power of mRNA-based Medicines **10:30** - Networking Refreshment Break	**10:00** - Delivering of Novel Cure: Unbridling the Power of mRNA-based Medicines **10:30** - Networking Refreshment Break	**10:00** - Delivering of Novel Cure: Unbridling the Power of mRNA-based Medicines **10:30** - Networking Refreshment Break
11:00	**11:00** - Trinucleotide Phosphoramidites: Synthons for Codon-based Gene Synthesis and Blockmers for Oligonucleotide Assembly **11:30** - Nanostar Sieving for Liquid Phase Synthesis of Oligonucleotides	**11:00** - Clinical Development of CXCL12 Inhibiting L-RNA Aptamer NOX-A12 (Olaptesed Pegol) **11:30** - The Oligonucleotide Agent BC 007 for Neutralization of Pathogenic Agonistic Autoantibodies Directed Against ß 1-Adrenoceptors in Heart Failure Patients – Up-date with Very First Phase II Data	**11:00** - Applying New Imaging Modalities to the ADME of ASO and Peptide Drugs **11:30** - Targeted Drug Delivery to the CNS and Peripheral Tissues Using the VECTrans® Innovative Technology	**11:00** - Applying New Imaging Modalities to the ADME of ASO and Peptide Drugs **11:30** - Targeted Drug Delivery to the CNS and Peripheral Tissues Using the VECTrans® Innovative Technology	**11:00** - Applying New Imaging Modalities to the ADME of ASO and Peptide Drugs **11:30** - Targeted Drug Delivery to the CNS and Peripheral Tissues Using the VECTrans® Innovative Technology
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SCHEDULE

MAIN CONFERENCE DAY 3 - 15/11/2019

TIME	OLIGONUCLEOTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	OLIGONUCLEOTIDE DISCOVERY, PRECLINICAL AND CLINICAL	PEPTIDE CHEMISTRY, MANUFACTURING AND CONTROLS	PEPTIDE DISCOVERY, PRECLINICAL AND CLINICAL	MRNA THERAPEUTICS AND CRISPR THERAPEUTICS
13:00	**13:40** - Chairperson's Remarks **13:45** - Clinical Development of Tivanisiran, A siRNA for the Treatment of Dry Eye Disease	**13:40** - Chairperson's Remarks **13:45** - Clinical Development of Tivanisiran, A siRNA for the Treatment of Dry Eye Disease	**13:40** - Chairperson's Remarks **13:45** - Control Strategies and Analytical Test Methods for Peptide-Conjugates	**13:40** - Chairperson's Remarks **13:45** - Control Strategies and Analytical Test Methods for Peptide-Conjugates	**13:40** - Chairperson's Remarks **13:45** - Strategies for Translocation Reduction during CRISPR/Cas Multiplexing
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15:00			**15:15** - Close of TIDES Europe 2019	**15:15** - Close of TIDES Europe 2019	**15:15** - Nanocarrier for CRISPR Gene Editing and mRNA-mediated Tumor Suppressor Rescue **15:45** - Close of TIDES Europe 2019