

EuroPEPTIDES: Part of TIDES Europe

7 - 10 November 2017 • Austria Trend Eventhotel Pyramide • Vienna

**INCREASE HALF-LIFE AND MINIMISE
MANUFACTURING COSTS THROUGH
NEW DATA, CASE STUDIES AND
TECHNOLOGIES FOR PEPTIDE
THERAPEUTICS**

**Industry Experts Present
Innovative Strategies To
Fast-Track Product Development**



**Purification of Synthetic Peptides
by Reversed Phase Chromatography**

Mathias Schaffrath

Sanofi, *Germany*



**Development of Liposomal
Formulations for Intracellular
Delivery of Therapeutic Peptides**

Alice Gaudin

IPSEN Innovation



**Lessons Learnt from Peptide Therapeutics
in Preclinical and Clinical Development**

Pernille Tofteng Shelton

Zealand Pharma, *Denmark*

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Gain the Science, Technologies and Contacts You Need to Accelerate Peptide Therapeutics to Market



300+

PEPTIDE AND OLIGONUCLEOTIDE SCIENTISTS AND EXECUTIVES

Fast-track your peptide research to the clinic and beyond by collaborating with leading pharma, biotechs, academia and solution providers from Europe, North America and Asia.



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CASE STUDIES AND NEW DATA PRESENTATIONS

Apply best practices and lessons learnt from industry leaders working across the entire spectrum of peptide development and production.



30+

LEADING EXHIBITORS

Connect with leading manufacturing, technologies, and service providers to drive your promising therapeutic towards commercial success.

**NEW
THIS
YEAR**

- New Vienna location after being in Berlin the past 4 years
- 85% of the 2017 speakers are new to the EuroPEPTIDES programme
- New industry case studies focused on:
 - ◆ Green Chemistry
 - ◆ Downstream Processing and Purification Techniques
 - ◆ Analytical Method Development and Validation
 - ◆ Analysis of Starting Materials in Peptide Manufacturing
 - ◆ Outsourcing Peptide Manufacturing
 - ◆ Peptide Vaccine Drug Development
 - ◆ Peptide Conjugates
- Create your own agenda – new 2,3 and 4-day pass options available



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Conference at-a-Glance

	EuroPEPTIDES	EuroTIDES	
	Tuesday 7 November 2017		
08:30 - 17:00	PEPTIDE DISCOVERY AND DRUG DEVELOPMENT STRATEGIES	WORKSHOP: OLIGONUCLEOTIDE THERAPEUTICS – DEFINING AND MANAGING CMC ACTIVITIES	
	Wednesday 8 November 2017 <i>Exhibit Hall Open</i>		
08:00 - 10:45	PRECLINICAL & CLINICAL DEVELOPMENTS, DELIVERY & FORMULATION	OPENING PLENARY SESSION	
11:30 - 17:00		Track One: Delivery & R&D Strategies	Track 2: Manufacturing and Scale Up Strategies
	Thursday 9 November 2017 <i>Exhibit Hall Open</i>		
08:50 - 10:15	PROCESS DEVELOPMENT, MANUFACTURING AND CMC	OPENING PLENARY: CRISPR AND GENOME EDITING APPLICATIONS	
10:45 - 17:00		Track One: Non-Clinical, Preclinical and Clinical Development	Track 2: CMC and Analytical Methods
	Friday 10 November 2017		
08:30 - 16:00	ANALYTICAL AND BIOANALYTICAL STRATEGIES FOR PEPTIDE DEVELOPMENT AND MANUFACTURING	mRNA THERAPEUTICS	

Tuesday 7 November 2017:

PEPTIDE DISCOVERY AND DRUG DEVELOPMENT STRATEGIES

08:30 *Registration*

09:00 **Chairperson's Opening Remarks**

09:10 **Phage Selection of High-Affinity Ligands Based on New Cyclic Peptide Formats**

Bicyclic peptides offer an attractive modality for the development of therapeutics. They can be developed by a procedure based on phage display in which linear peptides on the tip of phage are chemically cyclized prior to affinity selection. In my talk, I will present new chemical peptide cyclization strategies that we had developed recently and their application for the phage selection of high-affinity ligands.

Sangram Shivdas Kale, Postdoctoral Researcher, **Ecole polytechnique fédérale de Lausanne (EPFL), Switzerland**

09:50 **Spotlight Presentation: Peptide Selection Technologies**

If you are interested in giving this presentation please contact perri.lucatello@knect365.com

10:30 *Morning Coffee Break*

11:00 **Multi-Targeting Peptides for the Treatment of Obesity and Type 2 Diabetes**

Anish Konkar, Cluster Head, GI Endocrinology & Obesity, **Sanofi-Aventis Deutschland GmbH**

11:40 **Next Generation Peptide Drug Development Strategies – Feedback from GSK**

Graham Simpson, Head of Therapeutic Peptide Chemistry Performance Unit (CPU), **GlaxoSmithKline**

12:20 **Carbon-Carbon Bond Formation on Peptides**

Carbon-carbon bond construction is the basis for all of organic chemistry, given the multifunctional nature of peptides and the limited solubility of peptides in typical organic solvents, has limited the amount of carbon-carbon bond forming reactions that currently are being used routinely in combination with peptide chemistry and only a few examples exist on ligating peptide fragments based on Carbon-Carbon bond formation. The talk will focus on our work using organic catalysis to form Carbon-Carbon bonds on bioactive peptides.

Janos Kodra, Principal Scientist, Protein & Peptide Chemistry, **Novo Nordisk A/S, Denmark**

13:00 *Lunch*

14:00 **Targeting Protein-Protein Interactions with Peptide-Based Inhibitors**

Protein-protein interactions (PPIs) are essential to vital cellular processes, and serve as potential targets for therapeutic intervention. We are particularly interested in the PPIs between integral membrane proteins and their intracellular protein partners. We have developed peptide-based inhibitors of the PSD-95/glutamate receptor interaction, by exploiting that PSD-95 contains a tandem PDZ1-2 domain. So we designed and synthesized dimeric peptides with low nanomolar affinities, and have demonstrated that these ligands are potential treatment for ischemic stroke. For the same PPI, we examined the importance of backbone hydrogen bond by employing amide-to-ester mutations in peptide ligands and proteins. Finally, we have exploited the principle of dimeric peptide-based ligands to perturb the PPI between the scaffolding protein gephyrin and glycine/GABAA receptors. Most recently we have developed high affinity, cell-permeable peptides and demonstrated how these can modulate receptors and used to label synapses.

Kristian Strømgaard, Professor, Center for Biopharmaceuticals, **University of Copenhagen, Denmark**

14:40 **Macrocyclic Peptides – Powerful Therapeutic Modalities for Challenging Targets – From Discovery to Clinics**

Polyphor's drug discovery platform will be presented and exemplified with 2 showcases:

POL6014 a potent and selective human neutrophil elastase (HNE) inhibitor for chronic treatment in cystic fibrosis and potentially other rare lung diseases. POL6014 is currently in Phase 1b clinical trials and is administered topically (aerosol).

POL7080 a pathogen specific (*Pseudomonas aeruginosa*) antimicrobial drug with a novel mode of action. This macrocyclic peptide is about to enter Phase 3 clinical trials in Q1 2018 and is applied systemically (iv). POL7080 has spearheaded our efforts in antimicrobial drug discovery. It prompted a broad spectrum, gram negative OMPTA (Outer Membrane Protein Targeting Antibiotics) program, which has delivered very promising compounds currently in late stage lead optimisation.

Marc Thommen, Head of Chemistry, **Polyphor Ltd, Switzerland**

15:20 *Afternoon Coffee*

15:50 **Journey of Development for Peptide Therapeutics - Feedback from Amgen**

Les Miranda, Executive Director Research, Structural Biology, Molecular Modeling, & Hybrid Modality Engineering, Therapeutic Discovery, **Amgen Inc.**

16:30 **Tailor-Made Proteins for Biotechnology and Medicine Generated by Semisynthesis**

The ability to produce proteins in the laboratory and to change their structures and therefore their properties in a controlled fashion is of crucial importance in basic biological research, in biotechnology and increasingly in medical applications. I will discuss our efforts to use chemoselective ligation methods to assemble posttranslationally modified proteins from a combination of synthetic peptides and protein segments produced by expression (protein semisynthesis). Examples will include the semisynthesis of lipid-modified, membrane-attached proteins, of glycosylated peptides and proteins as well as proteins with non-enzymatic modifications.

Christian Becker, Head of the Institute of Biological Chemistry, **University of Vienna**

17:10 *End of Conference Day 1*

PRECLINICAL & CLINICAL DEVELOPMENTS, DELIVERY & FORMULATION

08:00 *Registration Morning Coffee and Networking*

08:50 **Chairperson's Opening Remarks**

Waleed Danho, Distinguished Research Leader and Consultant for Peptides, **Danho Associates Inc, USA**

09:00 **Feedback from Zealand Pharma - Lessons Learnt from Peptide Therapeutics in Preclinical and Clinical Development**

Pernille Tofteng Shelton, Senior Scientist, **Zealand Pharma, Denmark**

09:30 **Lessons Learnt from Peptide Therapeutics in Preclinical and Clinical Development**

10:00 **HER-2 B Cell Epitope Peptide-Based Cancer Vaccines and Combination Immunotherapies with EGFR, HER-3, IGF-1R, VEGF and a PD-1 Vaccine**

We have created and established a pipeline/ portfolio of validated B-cell peptide vaccines (HER-2, HER-3, HER-1, VEGF, IGF-1R and PD-1) against multiple receptor tyrosine kinases (RTK's) to expedite the development of new paradigm shifting cancer immune-therapies. We have translated two HER-2 B-cell epitopes peptide vaccines comprising epitopes designed to mimic the trastuzumab and pertuzumab binding epitopes to the clinic in an NCI-funded and FDA approved Phase 1/2b trial (NCT01376505). This presentation will detail our clinical trial and basic studies based on the development of combinatorial immunotherapeutic strategies that act synergistically to enhance immune-mediated tumor killing aimed at addressing mechanisms of tumor resistance for several tumor types.

Pravin Kaumaya, Professor, Vaccine Development/ Peptide & Protein Engineering Laboratory, **The Ohio State University Wexner Medical Center and the James Hospital and Comprehensive Cancer Center**

11:30 **Lessons Learnt from Peptide Therapeutics in Preclinical and Clinical Development: Lytix Biopharma**

Mette Husbyn, CMC Manager, **Lytix Biopharma, Norway**

12:00 **Cyclic Peptides: Translation into the Clinic**

If you are interested in giving this presentation please contact catherine.marshall@knect365.com

12:30 *Lunch*

14:00 **Development of Liposomal Formulations for Intracellular Delivery of Therapeutic Peptides**

Despite numerous intracellular therapeutic opportunities, less than 10% of peptides entering clinical trials have intracellular targets, due to inherent limitations such as poor cellular penetration and lysosomal degradation. With several formulations already on the market, liposomes have emerged as leading candidates for drug delivery, thanks to their versatility in terms of API formulation and therapeutic applications. Using a microfluidics platform, we developed several liposomal formulations of therapeutic peptides and investigated their intracellular entry. We demonstrated the possibility to efficiently load therapeutic peptides into liposomes, allowing effective delivery to tumor cells.

Alice Gaudin, Senior Scientist, **IPSEN Innovation**

14:30 **Overview of the Status and Challenges Associated with Delivery of Therapeutic Peptides to Improve Dosing Regimen (Extended PK/Half-Life) and Minimally Invasive Delivery**

Pradeep Dhal, Senior R&D Director, **Sanofi Global R&D, Genzyme Corporation - A Sanofi Company**

15:00 **Spotlight Presentation: Less Invasive Peptide Delivery**

If you are interested in giving this presentation please contact perri.lucatello@knect365.com

15:30 *Afternoon Coffee*

16:00 **Tailoring Peptides for Less Frequent Dosing by Endowing Them to Selectively Bind to Transthyretin**

Peptides hold great potential as anticancer agents. However, a major challenge impeding the widespread use of peptides is their poor pharmacokinetic profile, due to short circulation half-life. In this talk I will discuss our effort in developing a fundamentally new approach for enhancing the circulation half-life of peptides without affecting its biological potency.

Mamoun Alhamadsheh, Associate Professor of Pharmaceutical Chemistry, **University of the Pacific**

16:30 **Industry Case Study: Delivery Strategies for Targeting the Lymphatic System**

• Case study examples on sublingual delivery of peptides

Yves Decadt, Chief Executive Officer, **BioLingus**

17:00 *End of Conference Day 2 and Networking Dinner*

08:50 **Chairperson's Opening Remarks**

Jesper Lau, Vice President, Diabetes Protein & Peptide Chemistry, , **Novo Nordisk, Denmark**

09:00 **Comparison of Stepwise SPPS, Hybrid Method, and Native Chemical Ligation Approaches for Synthesis of the 51 Residue Ester Insulin Depsipeptide Chain**

Three distinct approaches were systematically explored for the efficient total chemical synthesis of human insulin lispro (Humalog). Native chemical ligation of peptide-thioester segments generated by Fmoc chemistry SPPS turned out to be optimal. The 3D-structure and correct disulfide pairing of the insulin lispro protein molecule were confirmed by high-resolution X-ray crystallography, and the synthetic protein was fully active in an insulin receptor binding assay

Stephen Kent, Professor, Department of Chemistry, **University of Chicago**

09:30 **Optimising Process Development and Large Scale Peptide Manufacturing: Process Modelling in Peptide Synthesis**

The development of models to predict the optimum conditions for solid phase peptide synthesis will be presented and scale up principals and pitfalls will be discussed. The effective deployment of novel modelling tools will be demonstrated for both the process development and commercial manufacture of peptides and the use of these tools to control unwanted side reactions will be examined.

Nigel Pitt, Senior Chemical and Process Development Chemist, **Ipsen Manufacturing Ireland Ltd.**

10:00 **Deletion Sequences In Fmoc SPPS - Root Cause Analysis and Prevention Strategies**

Frank Dettner , **Bachem**

10:30 *Morning Coffee and Networking*

11:15 **Divergent Protein Synthesis of Bowman-Birk Protease Inhibitors**

A divergent protein synthesis strategy was executed to effectively synthesize Bowman-Birk protease inhibitor analogues using native chemical ligation of peptide hydrazides. The crystal structure of a synthetic BBI analogue co-crystallized with a.chymotrypsin confirmed the correct protein fold and showed a similar overall structure to unmodified BBI in complex with a.chymotrypsin. Dynamic light scattering showed that C-terminal truncation of BBI led to increased self-association.

Christian Wenzel Tornøe, Principal Scientist, Department of Peptide & Protein Chemistry, **Novo Nordisk A/S, Denmark**

11:45 **Green Solid-Phase Peptide Synthesis (GSPPS)**

Recently, DMF, DCM and NMP, which are the most used solvents in Solid-Phase Peptide Synthesis have been classified as hazardous chemicals. Herein, we will discuss our work focused to substitute DMF by green solvents. The use of N-formylmorpholine, 2-MeTHF, isosorbide dimethyl ether, -valerolactone and ,,,,,-trifluorotoluene has been shown promising for SPPS.

Fernando Albericio , **University of KwaZulu-Natal (Durban, South Africa)** and **University of Barcelona (Barcelona, Spain)**

12:15 **Contribution of High Resolution Mass Spectrometry in Synthetic Peptides: Importance of Detailed Impurity Characterisation for Better Manufacturing**

Characterising large peptide impurities with confidence is a challenge. Affordable High resolution mass spectrometry (HRMS) systems, coupled to Ultra High performance Chromatography open new opportunities. This presentation will show, through real case studies, how in-house HRMS technology allows fast identification and enables chemists to build up knowledge for better manufacturing processes.

David Cosquer, Mass Spectrometry Specialist, **PolyPeptide SA, Belgium**

12:45 *Lunch*

14:00 **Preparative HILIC of Acidic, Difficult To Solubilise Synthetic Peptides**

HILIC is often overlooked as a method for peptide separations, due to concerns about peptide solubility and retention. We turned to HILIC for the purification of peptides consisting of only acidic and hydrophobic residues. For this class of peptides, yields in conventional RP-HPLC methodology are often very low and the HILIC method presented here has helped us overcome these problems.

Stephan Uebel, Head of Biochemistry Core Facility, **Max-Planck-Institute of Biochemistry**

14:30 **Purification of Synthetic Peptides by Reversed Phase Chromatography**

The purification of synthetic peptides (25-55 amino acids) is still a challenge. The unwanted by-products of these peptides are often peptides with only one wrong amino acid in the sequence. Therefore, the peptide and the by-products elute at the same time during the chromatographic separation. The reversed phase chromatography is in many cases the method of choice. Sometimes orthogonal reversed phase methods with two chromatographic steps and two different column selectivities are needed to increase the purity to more than 95%. Chromatographic experience, a thorough method development and up scaling is needed for successful separations. Partial automation of the process leads to a remarkable throughput, which is particularly important in the field of research.

Mathias Schaffrath, Group Head; IDD Chemistry/ Library Production, SM & Peptide Purification, **Sanofi, Germany**

15:00 **Spotlight Presentation**

A Representative from Zeochem

15:30 *Afternoon Coffee*

16:00 **PANEL DISCUSSION: Outsourcing of Peptide Drug Substance Manufacturing: What are the Driving Forces Behind this Industry Trend?**

Jesper Lau, Vice President, Diabetes Protein & Peptide Chemistry, **Novo Nordisk, Denmark**

17:00 **End of Conference Day 3**

Friday 10 November 2017:
**ANALYTICAL AND BIOANALYTICAL STRATEGIES FOR
PEPTIDE DEVELOPMENT AND MANUFACTURING**

08:30 *Registration*

09:00 **Chairperson's Opening Remarks**

Bruce Morimoto, Vice President, Scientific Affairs, **Celerion**,
USA

09:10 **Analytical and Bioanalytical Strategies for Peptide
Development and Manufacturing**

This multi speaker symposium will feature a series of case studies and group discussions focused on analytical and bioanalytical strategies for peptide development and manufacturing.

Some of the topics to be discussed include:

- Analytical method development, validation and transfer for peptide therapeutics
- Bioanalysis methods for understanding peptide biodistribution and absorption
- Development and application of new analytical methods and technologies for peptides
- Designing a holistic approach for starting material analysis to ensure high quality peptide APIs
- Characterisation techniques, impurity identification and predicting stability in drug development and manufacturing
- Process development and impurity tracking of complex peptide products
- Regulatory considerations for peptide impurities

Elisabeth Vey, Analytical Development Leader, **Ipsen**

Nicholas Pierson, Senior Scientist, **Merck**

**Enabling Technologies for Enhanced Analytical
Characterisation in the Development and
Manufacture of Peptide Therapeutics**

Synthetic peptide therapeutics of increasing complexity are being evaluated as a modality to access targets that are difficult to drug with small molecules. Analytical challenges associated with the development of peptide drug substances include resolving impurities that are chemically similar to the desired peptide API, and also involve the added need for biophysical characterisation. This talk covers recent advances in analytical technologies such as capillary electrophoresis coupled with mass spectrometry and ion mobility – mass spectrometry for analytical characterisation of peptides beyond purity and molecular weight determinations.

Nicholas Pierson, Senior Scientist, **Merck Sharp & Dohme**,
New Jersey, U.S.A.

**Effective Method Development – Strategies and
Lifecycle Management**

The presentation will describe the strategy for analytical method development for both commercial and development peptides. Various challenges posed by peptides are presented in the form of case studies. QbD is built into the method development phase by using a DoE approach to ensure rapid method validation.

Elisabeth Vey, Analytical Laboratory Leader, **Ipsen**, *France*

16:00 *End of Conference Day 4 and End of EuroPeptides 2017*

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▶ agenda

WORKSHOP • Tuesday 7 November 2017 • 10:00-16:30

10:00	Registration
10:30	Chairperson's Opening Remarks Thomas Rupp, Thomas Rupp Consulting, Germany
	Oligonucleotide Therapeutics – Defining and Managing CMC Activities
10:40	<p>Workshop will achieve the following: This highly strategic, introductory workshop will address cost of raw materials, manufacturing challenges and manufacturing activities to support a successful IND/IMPd submission towards initiation of clinical studies for oligonucleotide therapeutics. Participants will gain broad understanding of the regulatory CMC requirements and hurdles for oligonucleotide therapeutics in the US, Europe, Canada and Asia.</p> <p>Who Should Attend? Anyone interested in preclinical/clinical development of oligonucleotide therapeutics including scientists in R&D, manufacturing, quality control, quality assurance, project management, business development and regulatory affairs.</p> <p>General Workshop Overview:</p> <ul style="list-style-type: none"> • 4 talks, 45 minutes each presentation followed by 15 minutes discussion • 45 minute open discussion • Manufacturing: Synthesis chemistry overview • Manufacturing: Raw material cost development • Manufacturing: Considerations to manufacture clinical trial materials for phase 1 <p>Thomas Rupp, Thomas Rupp Consulting, Germany Kevin Fettes, Principal and Founder, FTS Pharma Consulting, LLC Yogesh Sanghvi, President, Rasayan Inc.</p>
16:30	Morning Coffee and Networking

MAIN CONFERENCE • Wednesday 8 November 2017:

8:00	Registration
8:50	Chairperson's Opening Remarks
	Opening Plenary Session
9:00	<p>SPINRAZA (Nusinersen) Approval: CMC Strategies and Lessons Learned Strategies for manufacturing and control, as well as CMC lessons learned from recent regulatory approval(s) of SPINRAZA (nusinersen) will be presented. Firoz Antia, Director, Antisense Oligonucleotide Process Development and Manufacturing, Biogen</p>
9:35	<p>RNAi Therapeutics in Human Disease Alnylam has advanced two delivery platforms for RNA interference (RNAi) based human therapeutics for liver-based disease molecular targets. The first one is lipid nanoparticles (LNPs) formulation containing siRNAs used for intravenous administration. The second one, in which siRNAs are conjugated to trivalent GalNAc sugar to target asialoglycoprotein receptor (ASGPR) of hepatocytes are emerging as a potential new class of medicine supporting a broad clinical pipeline across multiple therapeutic targets by subcutaneous administration. Furthermore, we have been able to continuously optimize the siRNA chemical modifications and design resulting in the Enhanced Stabilization Chemistry (ESC) platform exhibiting improved efficacy and extended duration lasting for several months. Our progress and therapeutic applications will be presented. Muthiah (Mano) Manoharan, Ph.D., Senior Vice President of Drug Discovery, Alnylam Pharmaceuticals, Inc.</p>
10:10	<p>Regulatory Authorities' Views and Expectations for Oligonucleotides This presentation will provide an overview about the regulatory landscape for oligonucleotides. The presentation will address the control strategy for synthetic oligonucleotides. Regulatory requirements for clinical trials and marketing authorisation applications will be highlighted. Experiences from recent regulatory submissions will be discussed. Since the early and open communication with Regulatory Agencies can significantly reduce time to market for a new drug product ways of interaction are exposed. Rene Thurmer, Deputy Head, Unit Pharmaceutical Biotechnology, BfArM - Federal Institute for Drugs and Medical Devices (awaiting final confirmation)</p>
10:45	Morning Coffee and Networking

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	Track One: Delivery & R&D Strategies	Track 2: Manufacturing and Scale Up Strategies
11:30	<p>Advances In Targeted Delivery Of Oligonucleotides Beyond The Liver</p> <p>AstraZeneca has in collaboration with IONIS Pharmaceuticals developed novel targeting approaches to enable specific delivery of oligonucleotide to cells other than hepatocytes and that are of interest in cardio-metabolic diseases. This talk will address some of the strategies and platforms developed to enable discovery and development of novel homing ligands that can be conjugated to antisense oligonucleotides for targeted delivery to pancreatic beta cells and that show efficient knockdown of genes in vitro and in vivo.</p> <p>Shalini Andersson, Head of Drug Metabolism & Pharmacokinetics, Cardiovascular and Metabolic Diseases, AstraZeneca</p>	<p>Improving Process Development in the Scale Up and Large Scale Manufacturing of Oligonucleotides</p> <p>Doug Brooks, VP CMC, Miragen</p>
12:00	<p>COMPACT: A Public-Private Partnership to Develop Novel Delivery Systems for Biopharmaceuticals</p> <p>COMPACT is a collaboration between pharmaceutical industry and academia sponsored by the EU Innovative Medicines Initiative to collectively work on the development of delivery strategies and systems for biopharmaceuticals as well as on the development of advanced models to study the transport of such delivery systems over biological barriers. In this presentation an overview will be given of the main activities and accomplishments of this consortium.</p> <p>Enrico Mastrobattista, dept. Pharmaceutics, Utrecht University, The Netherlands</p>	<p>Update on AJIPHASER of Oligonucleotide Synthesis for Large Scale Manufacturing</p> <p>We have reported the practical synthetic method AJIPHASE® which is a powerful tool for large scale manufacturing using olution-phase approach. The efficacy of AJIPHASE® has been proven with the successful synthesis of various oligonucleotides and with the same impurity profiles as it of solid-phase synthesis. This presentation will describe the technical updates on AJIPHASE® in detail and impurity analyses using some track records.</p> <p>Daisuke Takahashi, Senior Principal Researcher, Research Institute for Bioscience Products & Fine Chemicals, AJINOMOTO Co., Inc, Japan</p>
12:30	<i>Lunch</i>	
14:00	<p>Peptide-PMO Conjugates For The Enhanced Delivery And Treatment Of Neuromuscular Diseases</p> <p>We developed over previous years a series of Arg-rich peptides called Pip as PMO conjugates for boosting activity in mouse models in targeting pre-mRNA in Duchenne muscular dystrophy (exon skipping) and spinal muscular atrophy (exon inclusion). Pip-PMOs showed more than 100 times the activity over naked PMO in muscles of the DMD mdx mouse, including heart. Considerable extension of life was seen following systemic injection of a Pip-PMO into SMA mouse pups as well as high exon inclusion in brain and spinal cord of adult mice, providing strong evidence for crossing of the blood-brain barrier. We have now developed 3 further novel Arg-rich peptide classes as PMO conjugates for DMD systemic treatment as well as one novel class for strong activity in SMA mouse brain and spinal cord following intravenous delivery. All these novel peptide classes (D-PEPs) maintain the activity of Pip-PMOs but show improved tolerability and they form the basis of our current aims to develop enhanced activity peptide- PMOs for clinical treatment of DMD and SMA, as well as for myotonic dystrophy.</p> <p>Mike Gait, MRC Programme Leader, MRC Laboratory of Molecular Biology, Cambridge, United Kingdom</p>	<p>The Journey of Alicaforsen towards Commercialisation – Manufacture and Treatment</p> <p>Atlantic Healthcare is an emerging trans-Atlantic pharmaceutical company with a core focus on gastrointestinal disorders including Inflammatory Bowel Disease (IBD). Atlantic's lead product Alicaforsen enema is progressing through pivotal phase 3 clinical trial for the treatment of IBD, Pouchitis, due to read out 1H 2018. Alicaforsen is an antisense oligonucleotide, licensed from Ionis Pharmaceuticals, Inc., which provides the potential for the treatment of inflammation via multiple delivery formulations. Alicaforsen has been granted Orphan Drug designation by the FDA and EMA and a letter of Fast Track for the treatment of Pouchitis in recognition of the unmet medical need. The rolling NDA submission is underway with the non-clinical data section already submitted. Preparations for the CMC section, are well underway. The original API manufacturing process was developed over 12 years ago. Incorporating new approaches, Atlantic and Nitto Avecia have developed a new manufacturing process with process validation batches about to start. This presentation will describe the journey of alicaforsen through clinical and process development to provide an exciting new therapy for patients in need of a new treatment option for their condition.</p> <p>Janette Thomas, Director of International Operations, Atlantic Healthcare plc</p> <p>Mike Webb, Vice President, Manufacturing, Atlantic Healthcare plc</p>

	Track One: Delivery & R&D Strategies	Track 2: Manufacturing and Scale Up Strategies
14:30	<p>Development Of Novel Therapies Using Advanced GalNAc-siRNA Technology</p> <p>Silence Therapeutics uses RNA interference (RNAi) technology to develop new generation of drugs to treat serious human diseases with unmet needs. Conjugation of N-acetylgalactosamine (GalNAc) moieties to the RNAi triggers (siRNA) renders them ability to be delivered to and downregulate genes specifically in hepatocytes. We will present the current status of company's therapeutic pipeline based on proprietary GalNAc-siRNA technology.</p> <p>Dmitry Samarsky, CSO, Silence Therapeutics, 5</p>	<p>Sustainability of Oligonucleotide Processes</p> <p>This presentation will cover AstraZeneca's definition of sustainability and how oligonucleotide manufacturing processes impact the environment. A comparison will be made to the environmental impacts of small molecule API manufacture. Finally, potential process development opportunities to improve the sustainability of oligonucleotide processes will be discussed.</p> <p>Anna Watson, Scientist (Process Engineer), Pharmaceutical Technology and Development, AstraZeneca, UK</p>
15:00	<p>Enhancing ASO Potency in Extra-Hepatic Tissues</p> <p>Recent advances in targeted delivery have greatly enhanced the potency of oligonucleotide therapeutics for suppressing gene expression in hepatocytes. We have explored strategies to enhance potency of oligonucleotide therapeutics in extra-hepatic tissues such as muscle, endocrine organs and in lymphocytes, which will be presented.</p> <p>Punit Seth, Vice President, Ionis Pharmaceuticals</p>	<p>Fully-Scalable, cGMP Production of Oligonucleotide-Containing Nanoparticles</p> <p>Polymun has unique know-how and technology for the development and manufacturing of liposomal formulations. Our proprietary crossflow injection technology is based on the solvent injection method and over the past 15 years it was used to formulate various nanoparticle formulations intended for clinical trials.</p> <p>Andreas Wagner, Head Liposome Technology, Polymun Scientific Immunbiologische Forschung GmbH</p>
15:30	<i>Afternoon Coffee</i>	
16:00	<p>How Phosphorothioate Oligonucleotides Traffic from the Endosome to the Cell Nucleus</p> <p>After internalisation into cells under the conditions of gymnosis (i.e., no transfection reagents or carriers), splice-switching oligonucleotides (SSOs) are often deeply trapped in cytoplasmic vesicular structures, and often only poorly perform their nuclear function. We have examined the formation of a protein/SSO complex that we believe traffics the SSO to the nucleus, and have identified two distinct, yet synergistic methods, for augmenting the nuclear localisation of an SSO after delivery by gymnosis.</p> <p>Cy Stein, Professor of Medicine, Experimental Pharmacology, and Cellular and Molecular Biology, City Of Hope, USA</p>	<p>SPOTLIGHT PRESENTATION: Innovative Process Development, Scale Up and Synthesis for Oligonucleotide Therapeutics to Reduce Cost of Goods</p> <p>If you are interested in giving this presentation please contact perri.lucatello@knect365.com</p>
16:30	<p>Nucleic Acid Mimetics As Broad-Spectrum Drug Discovery Platform</p> <p>We will present initial results related to our efforts to develop a new class of oligonucleotide analogues as potent and specific therapeutic agents. The main focus was at structurally diverse DNA and RNA analogues, namely, new sugar ring modified oligonucleotide -N3'-P5'-(thio)-phosphoramidates and their conjugates with tissue targeting ligands. These compounds were designed, synthesized and evaluated in vitro and in vivo primarily as antisense and siRNA agents, as well as 3-D RNA structure remodelling molecules.</p> <p>Sergei Gryaznov, Senior Director, Oligonucleotide Center of Excellence, Part of the Janssen Pharmaceutical Companies of Johnson & Johnson</p>	<p>PANEL DISCUSSION: Ensuring Raw Materials Supply Chain Quality for Oligonucleotides</p> <ul style="list-style-type: none"> • What control strategies are being used to ensure raw materials / starting materials quality? • What are critical and non-critical impurities? • Calculation strategies to calculate purity of APIs • How do suppliers ensure quality as larger batches are produced? • What are industry doing to ensure quality of starting materials? Are people doing their own testing as well as suppliers? • How to handle isomer impurities which can't be detected in products? • Regulatory feedback on the quality control requirements for raw materials: What is expected in terms of control of starting material and level of information included in the dossiers?
17:00	<i>End of Day 2 and Networking Dinner</i>	

8:50	Chairperson's Opening Remarks	
	Opening Plenary: CRISPR and Genome Editing Applications	
9:00	CRISPR Genome Editing: Considerations for Therapeutic Applications As we work to develop CRISPR-based medicines, Editas is similarly focused on developing enabling technologies that will allow us to deliver efficient and safe medicines. These include advancements to better assess specificity referred to as Uni-directional Targeted Sequencing (UDITAS™), novel compositions and methods for making covalently-coupled dual guide RNAs, and targeted insertion of DNA for efficient and specific gene correction. Cecilia Fernandez , Senior Director of Platform Strategy and Operations, Editas Medicine	
9:40	Thought Leaders Discussion: Overcoming the Delivery Challenges of Oligonucleotides with CRISPR <ul style="list-style-type: none"> • Evaluating in vivo delivery methods to get genome editing into the tissues and cells • Addressing in vivo delivery of oligonucleotides via genome editing • How best to deploy reagents and delivery of editing. • Oligonucleotide delivery using CRISPR technology • Chemical modification on RNA using CRISPR to improve delivery 	
10:15	<i>Morning Coffee and Networking</i>	
	Track One: Non-Clinical, Preclinical and Clinical Development	Track 2: CMC and Analytical Methods
10:45	A Sensitive In Vitro Approach To Assess The Hybridisation-Dependent Hepatic Liability Of High Affinity Single Stranded Gapmer Oligonucleotides I will present an in vitro screening approach, which strongly predicts the potential hepatic liability of LNA-modified single stranded oligonucleotides (SSOs). I will show data demonstrating that the in vitro assay accurately reflects recent in vivo findings related to the underlying mechanisms of SSO hepatotoxicity. Moreover, I will provide evidence that this kind of toxicity is 'preserved' in different cell types and even in different species suggesting good translatability of the in vitro results to animals and humans. Andreas Dieckmann , Senior Principal Scientist, Roche Innovation Center Basel, Switzerland	
11:15	Feedback from Roche Innovation Center Copenhagen A/S Troels Koch , VP & Head of Research, RNA Therapeutics, Roche Innovation Center Copenhagen, Denmark	Development of a Robust Analytical Control Strategy for the Manufacture of Liquid Oligonucleotide Drug Substance The control strategy includes the development and implementation of novel methods for antisense oligonucleotide analysis, including process analytical technologies (PAT), moving the control points upstream, phase appropriate validation of the analytical methods, minimizing redundant testing, and setting specifications for a liquid oligonucleotide drug substance. Where appropriate, Biogen's experience with the regulatory authorities will also be included. Jessica Stolee , Sr. Scientist, Biogen
11:45	Clinical Trials for SYL1001, a Novel Short Interfering RNA for the Treatment of Dry Eye Disease Sylentis has performed several Phase I and Phase II clinical trials to evaluate the safety and efficacy of SYL1001 eye drops on the treatment of dry eye disease. Phase II results showed excellent tolerability and significant improvement in the disease after 10 days of treatment compared to placebo. Additionally, dose response biodistribution studies demonstrated good correlation with clinical results. Anne Marie Bleau , Clinical Operations Manager, Sylentis SAU, Spain	Development and Application of an Analytical Control Strategy to a Commercial Product An insight into using a data and safety driven approach to define a commercial control strategy and working with CMOs and CROs to implement this strategy in the commercial environment. Utilising extensive experience within GSK of managing commercial products and developing robust control strategies, we have transferred the knowledge into an oligonucleotide space and this talk will look at the specific nuances when working with these molecules. Rachel Orr , Scientific Investigator, GSK, UK
12:15	Achieving HBsAg Loss with Nucleic Acid Polymers: Updates on Pharmacology, Toxicity and Restoring Functional Cure of HBV and HDV Infection NAPs have a unique ability to eliminate circulating HBsAg, allowing immunotherapy to restore functional control of HBV and HDV infection. Recent modeling of NAP effects in vitro and evolving pre-clinical and clinical data continue to advance the understanding of how NAPs work and their clinical impact against HBV and HDV infection. Andrew Vaillant , Chief Scientific Officer, Replicor, Inc.	Analytical Strategy for Improving Chromatographic Separation of Oligonucleotides by Denaturing HPLC/ UHPLC/UV Methods The use of nontraditional gradients in anion exchange HPLC and ion pairing reverse phase HPLC/ UPLC methods have the potential to address the challenges in separation of critical pairs in the denaturing analysis of oligonucleotides. Linear and nonlinear gradients will be compared to demonstrate the improvements in the chromatographic separation of the single strands and N-1 and N+1 sequence failures. Robyn Milburn , Senior AD Chemist, Analytical Development, NITTO DENKO AVECIA INC.
12:45	<i>Lunch</i>	

	Track One: Non-Clinical, Preclinical and Clinical Development	Track 2: CMC and Analytical Methods
14:00	<p>Targeting Transforming Growth Factor Beta 2 (TGF-β2) mRNA with ISTH0036 as Novel Therapeutic Intervention in Ocular Diseases</p> <p>Although anti-VEGF therapeutics represent a significant step forward in treating neovascular eye diseases, insufficient response to anti-VEGF treatments represents a significant clinical problem. Therefore, novel therapies with alternative molecular mechanisms of action are desirable. Here we report on preclinical investigations of ISTH0036, a LNA modified antisense oligonucleotide specifically targeting the sequence of TGF-β2 mRNA, in in vitro and in vivo models to obtain proof of concept for the advancement of ISTH0036 into clinical testing.</p> <p>Katja Wosikowski, Director Preclinical Operations, Isarna Therapeutics, Germany</p>	<p>Analytical Method Development and Validation for Oligonucleotide Therapeutics: Feedback from Intertek</p>
14:30	<p>Increasing the Efficacy of Cancer Immunotherapy using RNAi</p> <p>The development of cancer immunotherapy has greatly improved outcomes for a fraction of patients, but many tumors remain insensitive to these groundbreaking medicines. DCR-BCAT is a nanoparticle-formulated Dicer-substrate siRNA (DsiRNA), which targets CTNNB1 mRNA in tumors. In preclinical models of diverse cancer types, systemic administration of DCR-BCAT significantly improves response rates to immunotherapy. We will cover the mechanism by which this experimental RNAi therapeutic enables tumor regression and synergistic efficacy.</p> <p>Marc Abrams, Sr. Director Preclinical Research, Dicerna Pharmaceuticals</p>	<p>IPRP UPLC Method Optimisation for the Separation of Short- and Long-mer Impurities in Oligonucleotide Therapeutics</p> <p>Hilde van Hattum, Sr Scientist Characterisation & BioAnalytics, ProQR Therapeutics BV</p>
15:00	<p>Preclinical Development and Phase 1 Preparation for Oligonucleotide Therapeutics: STP705 CMC Experience and Beyond. Lessons Learned</p> <p>Preparing for IND/IMPd is a critical step for successful start of clinical development. Making a few batches of oligonucleotide is not the end of the process. We will review a few cases and give some sort of path to success based on several cases.</p> <p>Marc Lemaître, Independent Consultant</p>	<p>Spotlight Presentation: Current and Future Analytical Techniques Applied for Quality Control in Oligonucleotide Manufacturing</p> <p>If you are interested in giving this presentation please contact perri.lucatello@knect365.com</p>
15:30	<i>Afternoon Coffee</i>	
16:00	<p>Topically-Applied Antisense Spherical Nucleic Acid for the Treatment of Psoriasis</p> <p>We are developing a topical antisense oligonucleotide, called AST-005, which is targeted to tumor necrosis factor alpha mRNA for the treatment of chronic plaque psoriasis. Drug discovery and development efforts by Exicure revolve around the use of spherical nucleic acid (SNA) constructs, which are 3-dimensional arrangements of oligonucleotides where the nucleic acids are densely packed and radially oriented. This presentation will describe the development of AST-005 and its topical gel vehicle, the clinical trial-enabling nonclinical toxicology and drug disposition program and results, Phase 1 clinical trial progress, and overall regulatory strategy for the program.</p> <p>David Giljohann, CEO, Exicure</p>	
16:15	<p>Discovery and Development of microRNA Targeting Therapeutic Candidates for the Treatment of Hematological Malignancy and Pathological Fibrosis</p> <p>Dysregulation of the expression of certain microRNAs can lead to the development of self-reinforcing feedback loops that contribute to disease pathology. We believe that targeting such pathological microRNAs for therapeutic intervention may result in disease modifying therapies. We have advanced two product candidates into clinical trials; MRG-106, a LNA antimir® targeting microRNA-155 in hematological malignancies and MRG-201, a synthetic replacement (promiR) for microRNA-29 in pathological fibrosis. An overview of our latest clinical observations will be presented.</p> <p>William Marshall, President & CEO, miRagen Therapeutics, Inc.</p>	
16:30	<p>Immunologic Reshaping and Therapy of Cancer by Stimulating the Innate Nucleic Acid Sensor RIG-I</p> <p>Rigontec identified an optimized, synthetic oligonucleotide, designated RGT100, selective for RIGI. RGT100 activates the RIG-I pathway leading to the induction of Th1-dominated cytokines and immunogenic tumor cell death. Rigontec's RGT100 demonstrates strong anti-tumor activity in clinically relevant mouse tumor models, while bearing an advantageous safety profile. RGT100 has entered clinical evaluation in advanced cancer patients in Q1 2017.</p> <p>Jorg Vollmer, Chief Scientific Officer, Rigontec, Germany and USA</p>	
16:45	<p>Breaking the Immunosuppressive Tumor Microenvironment with Antisense Oligonucleotides</p> <p>Treatment of cancer with antisense oligonucleotides (ASOs) remains a big challenge. On the other hand, immunotherapy of cancer has emerged as a promising approach. Within oncology Secarna has focused on targeting immunosuppressive factors within the tumor microenvironment using modified ASOs. Exemplarily preclinical data will be presented that show the progress of Secarna's program targeting the immunosuppressive ectonucleotidase CD39.</p> <p>Frank Jaschinski, CSO, Secarna Pharmaceuticals GmbH & Co. KG, Germany</p>	
17:00	<i>End of Conference Day 3</i>	

8:30	<i>Registration</i>
9:00	Chairperson's Opening Remarks
mRNA Therapeutics	
9:10	Feedback from Moderna: Discovery and Development of mRNA Therapeutics Hari Pujar , Vice President, Technology Development & Manufacturing , Moderna Therapeutics
9:50	mRNA Manufacturing for Individualised Cancer Treatment Messenger (m)RNA is increasingly investigated as a platform technology for multiple therapeutic applications. The employment of mRNA to deliver the genetic information of antigens into antigen presenting dendritic cells offers new options for immunotherapies against cancer, especially in the context of individualized treatment. The presentation will deal with the GMP manufacturing of mRNA as well as approaches to overcome challenges of conducting an individualized clinical trial with an appropriate infrastructure setup. Part of the presentation will also be about first experiences from our clinical program. Christoph Kroener , Head of IVAC Mutanome Lead Structure, BioNTech RNA Pharmaceuticals GmbH
10:30	<i>Morning Coffee and Networking</i>
11:00	Feedback from CureVac: Discovery and Development of mRNA Therapeutics Thomas Schlake , Head of Enabling Technologies, CureVac AG, Germany
11:40	Strategies to Overcome the Delivery Challenges for mRNA If you are interested in presenting on this topic please contact catherine.marshall@knect365.com
12:20	<i>Lunch</i>
13:20	Towards mRNA Therapeutics for Skin Diseases Skin offers various opportunities with regard to development of mRNA-based therapeutics: diseases with validated molecular targets/attractive markets and direct access facilitating quantification of mRNA expression/clinical activity. mRNA is a new drug format capable of exceeding existing protein-based therapeutics. ACCANIS develops proprietary IVTmRNAs addressing validated targets for specific skin conditions. We systematically modified specific IVTmRNAs and tested the most interesting ones in skin explant systems varying formulation and delivery. Markus Mandler , CSO, Accanis Biotech
14:00	mRNA Analytical and CMC Strategies If you are interested in presenting on this topic please contact catherine.marshall@knect365.com
14:40	<i>Afternoon Coffee</i>
15:10	Interactive Group Discussion: Overcoming the Challenges in Developing mRNA Therapeutics <ul style="list-style-type: none"> • Discovery and development of mRNA therapeutics • Differences between mRNA and traditional oligonucleotides products • Preclinical and clinical development: Advancing mRNA products into the clinic • Strategies to overcome the delivery challenges for mRNA delivery • Formulation strategies • mRNA analytical and CMC strategies – Understanding and measuring key quality attributes for mRNA therapeutics • Optimising large scale manufacturing and scale up of mRNA Therapeutics • Regulatory pathways for mRNA therapeutics Hari Pujar , Vice President, Technology Development & Manufacturing, Moderna Therapeutics Christoph Kroener , Head of IVAC Mutanome Lead Structure, BioNTech RNA Pharmaceuticals GmbH Thomas Schlake , Head of Enabling Technologies, CureVac AG, Germany Markus Mandler , CSO, Accanis Biotech
16:10	<i>End of Conference Day 4 and End of EuroTides 2017</i>

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