

COST Action CA17103
Action Title: Delivery of Antisense RNA Therapeutics

Agenda Workshop WG2 WG3

11th-12th October 2019
MKG Chirurgie
Grosser Hörsaal/Large Lecture Hall, 2nd floor
Lindwurmstraße 2A 80377 Munich

JOINED WORKSHOP: MODEL SYSTEMS & SAFETY AND TOXICOLOGY

FRIDAY 11th October 2019

12:00 Cold lunch for pre-registered participants

13:00 Collection of accreditation & coffee

Signing of COST attendance sheets

13.30 Welcome to participants and introduction

Workshop and local organizers:

Topics: Task groups for writing SOPs and guidelines for toxicity screening

13.50 – 17.20 SESSION I: DELIVERY AND MODELS

13.50 Models and delivery of ASOs for skin diseases

Dr. Jeroen Bremer (University Medical Center Groningen, Netherlands)

14.10 Finding specific pancreatic beta cell surface proteins for imaging and delivery of ASOs

Prof. Decio Eizirik (ULB Center for Diabetes Research, Belgium)

14.30 Models to study therapeutic RNA-based molecules for lysosomal storage diseases and how to deliver them

Dr. Sandra Alves (Instituto Nacional de Saúde Dr. Ricardo Jorge, Portugal)

14.50 Alternative Splicing modulation as a novel treatment for Lung cancer

Dr. Jordi Hernández Ribera (Centre for Genomic Regulation, Spain)

15.10----- Coffee break -----



15.40 Delivery of Oligonucleotides to the Nervous System: do we still need to rack our brains?

Prof. Michela Denti (University of Trento, Italy)

16.00 How to use retinal organoids for translation of ASOs to the clinic

Prof. Michael Cheetham (University College London, United Kingdom)

16.20-17.20 Q&A - Brainstorming session I- with the panel of speakers

17.30-19.00 ELEVATOR PITCH/SHORT RESULTS SESSION

ROUND 1: TREATMENT OF DISEASES

17:35 Exon-skipping oligonucleotides restore functional collagen VI by correcting a common COL6A1 mutation in Ullrich Muscular Dystrophy (Sara Aguti, United Kingdom)

17:40 Potential antisense based therapy for PAH exon 11 skipping variants in phenylketonuria (Ainhoa Martinez, Spain)

17:45 Therapeutic Evaluation of Antisense Oligonucleotides based Multiple Exon Skipping Strategies in a Duchenne Muscular Dystrophy Patient (Arun Shastri, India)

17:50 First steps towards a comprehensive comparison of chemical modification-dependent AON biodistribution in retinal models (Irene Vazquez-Dominguez, the Netherlands)

17:55 ASOs targeting lncRNAs with oncogenic potential in hepatocellular carcinoma Puri Fortes, Spain)

18:00-18:15: Q&A Round 1

ROUND 2: DELIVERY

18:15 Targeted liposomal drug delivery in prostate cancer via ssDNA aptamer (Olcay Boyacioglu, Turkey)

18:20 Complexation of Bioactive Peptide-Oligo Nanostructures as Therapeutic Agents (Deniz Sezlev Bilecen, Turkey)

18:25 A new platform for the direct profiling of nucleic acids in biofluids (Simone Destassis, Italy)

18:30 Development of beta cyclodextrin (BCD)-templated “smart” polymeric nanoparticles for siRNA delivery (Omer Aydin, Turkey)



18:35 Development of peptide nanofibers as bioactive carriers (Gulcihan Gulseren, Turkey)

18:40-18:55 Q&A Round 2

19.30 The Bavarian Experience at Lindwurmstueberl (only for pre-registered participants)

SATURDAY 12th October 2019

9.00 Collection of accreditation & Coffee

Signing of COST attendance sheets

9.10 – 12.00 SESSION II: SAFETY AND TOXICOLOGY OF SYSTEMICALLY ADMINISTERED ASO

9.10 Introduction lecture about ASO chemistries, PK and the different type of ASO-mediated toxicities

Dr. Patrik Andersson (AstraZeneca, Sweden)

9.40 – 12.00 SAFETY PART 1. PROTEIN BINDING EFFECTS

9.40 ASO-mediated Immune activation (Overview presentation of immunostimulatory mechanisms) Dr. Art Krieg (Checkmate Pharmaceuticals, Cambridge, USA)

10.00 Identification of Proinflammatory ASOs: Lessons learned from thousands of ASO screen

Dr. Padma Narayanan (Ionis Pharmaceuticals, Carlsbad, USA)

10.20----- Coffee break -----

10.40 ASO mediated complement activation / thrombocytopenia

Dr. Padma Narayanan (Ionis Pharmaceuticals, Carlsbad, USA)

11.00 Q&A - session Safety Part 1 - with the panel of speakers



12.00 – 16.00 SAFETY PART 2. TOXICITY IN HIGH EXPOSURE ORGANS

12.00 In vitro safety screening for liver and kidney toxicity: “In vitro Assays capturing toxicity risks”

Dr. Sabine Sewing (Roche, Basel, Switzerland)

12.20 Histopathology analysis of liver and kidney: identification of typical lesions induced by ASO

Dr. Pierluigi Fant (Charles River, Lyon, France)

----- 12.40 Lunch break -----

14.00 Safety Evaluation of 2'-Deoxy-2'-Fluoro-Modified Nucleotides in GalNAc-siRNA Conjugate

Dr. Mark Schlegel (Alnylam Pharmaceuticals, Cambridge, USA)

14.20 Q&A - session Safety Part 2- with the panel of speakers

14.50 Brainstorming, how to set up guidelines and SOPs

Define task groups and timeline to write guidelines and SOPs

15.50 Closing remarks and Coffee

MANAGEMENT COMMITTEE MEETING

16.20-19.00

Only management committee members will be invited to this meeting

