



Regulatory and scientific virtual conference on RNA-based medicines

2 February 2023, 09:00 – 16:30 (CET)

Speakers' biography



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Welcome and introduction



Emer Cooke

European Medicines Agency (EMA)

Emer Cooke is as of November 2020 the Executive Director of the European Medicines Agency. She also takes the role of Chair of the International Coalition of Medicines Regulatory Authorities (ICMRA) for a term of 2 years. She was the Director responsible for all medical product-related regulatory activities at the World Health Organization in Geneva between November 2016 and November 2020. In this role, Ms Cooke was responsible for leading WHO's global work on regulation of health technologies (medicines, vaccines, diagnostics, vector control products and devices), coordinating the regulatory teams (prequalification, regulatory systems strengthening, and safety), and working with member states and international partners to assure the quality, safety and efficacy of appropriate health technologies. Ms. Cooke is a pharmacist with Masters degrees in Science and Business Administration from Trinity College Dublin. She has over 30 years' experience in international regulatory affairs and spent 14 years (2002 to 2016) in management positions at EMA as Head of Inspections and Head of International Affairs respectively. From September 1998 to July 2002, she worked in the Pharmaceuticals unit of the European Commission.



Steffen Thirstrup

European Medicines Agency (EMA)

Steffen Thirstrup has been the Chief Medical Officer at EMA since June 2022. He is a board-certified specialist in clinical pharmacology and therapeutics, holds a PhD in pharmacology, and is an affiliated professor at Copenhagen Centre for Regulatory Science (CORS) at Copenhagen University, Faculty of Health. Steffen has a broad clinical background in general internal medicine and respiratory medicine, and more than 20 years' experience from working in the EU regulatory system both at a national competent authority (DK), as member of EMA committees (CHMP and CAT) and working parties, as well as a regulatory consultant.



Sol Ruiz

Agency of Medicines and Medical Products (AEMPS), ES

Sol Ruiz is Head of Biologics, Biotechnology and Advanced Therapies at AEMPS. She has a PhD in Biology (Immunology) and is the chair of the BWP (Biologics Working Party) at the EMA (European Medicines Agency) since March 2014. She is also a member of the CAT (Committee for Advanced Therapies) and a co-opted member of the CHMP (Committee for Human Medicinal Products) since 2007.

Session 1 - State of the art of RNA technologies



Falk Ehmann

European Medicines Agency (EMA)

Falk Ehmann is Senior scientific officer in the Regulatory Science & Innovation Task Force (TRS) responsible for chairing the EMA's Innovation Task Force (ITF) and Business Analysis and Forecasting activities. Falk is a medical doctor specialised in clinical pharmacology with a PhD in Experimental Medicine and a degree in International and European law. His special interests include -omics, personalised medicines and nanotechnologies promoting regulatory science via research and development of innovative substances, methodologies and technologies.



Tal Zaks

Orbimed, US

Tal Zaks, M.D., Ph.D., is a Partner with OrbiMed. Dr. Zaks was recently the Chief Medical Officer at Moderna, where he led the development of the company's COVID-19 vaccine and other key programs. Dr. Zaks also serves as a non-executive director of Teva and Adaptimmune, and is an adjunct Associate Professor of Medicine at the University of Pennsylvania. Previously, Dr. Zaks held senior leadership positions in drug development at major pharmaceutical companies, including Sanofi and GlaxoSmithKline. Dr. Zaks received his M.D. and Ph.D. from the Ben Gurion University and holds a Ph.D., honoris causa from the Bar Ilan University.



Annemieke Aartsma-Rus

Leiden University Medical Center, NL

Prof. Dr. Annemieke Aartsma-Rus is a professor of Translational Genetics at the Department of Human Genetics of the Leiden University Medical Center (LUMC, the Netherlands). She played an important role in the development of antisense mediated exon skipping for Duchenne muscular dystrophy, a topic she has been working on since her PhD research (2000-2004). In 2020 she co-founded the Dutch Center for RNA Therapeutics (DCRT), a non-for-profit academic collaboration aiming to develop clinical treatment with exon skipping therapies for eligible patients with unique mutations. In 2022 she became a board member of the N-of-1 collaborative (N1C), a global umbrella organization aiming to facilitate development of antisense oligonucleotide therapies for patients with very rare, eligible mutations. She has published over 230 peer-reviewed papers, 11 book chapters and 15 patents. In 2011 she received the Duchenne Award from the Dutch Duchenne Parent Project in recognition of her dedication to the Duchenne field. In 2020 she received the Black Pearl Science Award from Eurordis for her work in educating patients. In 2021 she received the Ammodo Science Award for

her contribution to developing exon skipping therapies for Duchenne and the outstanding achievement award from the Dutch Society of Gene and Cell Therapy for her work.

She is chair of the TREAT-NMD Advisory Committee for Therapeutics (TACT), Chair of the Dutch Center for RNA Therapeutics, vice-chair of COST Action CA17103 (Delivery of antisense RNA therapies), board member of the N-of-1 Collaborative, was President of the Oligonucleotide Therapeutics Society (2019-2021), and was Chair of the TREAT-NMD executive committee (2013-2016 and 2019-2020). She has been instrumental in setting up training courses, e.g. TREAT-NMD Duchenne masterclasses, the TREAT-NMD/EURO-NMD translational summer school and a COST training school on oligonucleotide therapy development (2020). She is a member of the Therapies Scientific Committee of the International Rare Disease Research Consortium (IRDiRC). She is co-editor in chief of Nucleic Acid Therapeutics, and serves on multiple editorial boards, e.g. Journal of Neuromuscular Diseases (associate editor), Molecular Therapy and Therapeutic Advances in Rare Diseases.

Session 2 - Application of RNA technologies: CMC



Brian Dooley

European Medicines Agency (EMA)

Brian Dooley is a senior pharmaceutical quality specialist in the Pharmaceutical Quality Office of EMA. Brian has been at EMA since 2016, working mainly on centralised marketing authorisations and scientific advice, and supporting the development of scientific guidelines by the CHMP, QWP and BWP. Prior to EMA, Brian worked as a pharmaceutical assessor in the Irish medicines regulator (IMB/HPRA). He is a pharmacist by training and holds an M.Sc. in Pharmaceutical Medicine from Trinity College Dublin. His areas of interest include: lifecycle management, quality risk management, assessment-inspection interface, synthetic peptides, oligonucleotides, mRNA technology and sterilisation processes.



René Thürmer

Federal Institute for Drugs and Medical Devices (BfArM), DE

Dr René Thürmer received his diploma in chemistry and his Ph.D. in biochemistry from the University of Tübingen. He joined the BfArM (Federal Institute for Drugs and Medical Devices, Bonn, Germany) in 2000. He currently serves as a CMC reviewer and is Deputy Head of the Unit Pharmaceutical Biotechnology. His experience is in the field of formulation, manufacture and control of medicinal products, in particular in the field of oligonucleotides, peptides, proteins, liposomes, sustained release polymer drug products, depot formulations, polymer-conjugated drug products, natural and synthetic surfactants, nanomedicine and others. He is a member of the EMA Quality Innovation Group (QIG).



Daniel Capaldi

Ionis Pharmaceuticals, Inc., US

Daniel is Vice President, Analytical and Process Development at Ionis Pharmaceuticals, Inc. He received a Ph.D in chemistry from King's College, London. He was a Cancer Research Campaign fellow at King's for two years before moving to work with Prof. R. F. Schinazi in Atlanta, Georgia. In March 1996, Daniel joined Ionis Pharmaceuticals, Inc. in Carlsbad, California as a process chemist where he spent four years working on process improvements in large-scale oligonucleotide synthesis. In his current position as Vice President, Analytical and Process Development, Daniel is contributing to the development of antisense therapeutics and has responsibility for a variety of process chemistry and CMC functions including supply chain improvements and process optimization, analytical method development and validation, release and stability testing and impurity characterization. Daniel was a lead author of the marketing applications for four approved oligonucleotide therapeutics [mipomersen (KYNAMRO™), nusinersen (SPINRAZA™)], inotersen (TEGSEDI™) and volanesorsen (WAYLIVRA™). Daniel has co-authored over 30 papers and patents in the field of oligonucleotide chemistry.



Pawel Widomski

BioNTech SE, DE

Pawel works at BioNTech's Global Regulatory CMC Infectious Disease team and leads the lifecycle management of the Comirnaty vaccines. He brings 12+ years of global CMC regulatory experience for both development and marketed products of numerous biological and small molecules and various dosage forms in various indications, including cardiology, oncology and infectious diseases.



Lubomir Nechev

Alnylam Pharmaceuticals, Inc., US

Lubomir Nechev, Ph.D. is the SVP CMC Development of Alnylam Pharmaceuticals. Dr Nechev is one of the pioneers in the CMC aspects of RNAi therapeutics. He has worked at Ribozyme Pharmaceuticals (RPI), the Nucleic Acids Synthesis unit of Transgenomic, Inc., and has joined Alnylam in March 2004. In the last 19 years, Dr. Nechev has led the development and implementation of the CMC strategy for siRNA therapeutics used in five approved products – ONPATTRO®, GIVLAARI®, OXLUMO®, Leqvio® (Novartis), and AMVUTTRA™. The first of them (ONPATTRO®) is also the first approved lipid nanoparticle (LNP) formulated siRNA and the second one (GIVLAARI®) is the first approved GalNAc-conjugated siRNA. Dr. Nechev received his Ph.D. degree in Organic Chemistry from St. Kl. Ohridski University, Sofia, Bulgaria and completed his post-doctoral training at the Department of Chemistry and Center in Molecular Toxicology at Vanderbilt University, Nashville, Tennessee.



Andreas Kuhn

BioNTech SE, DE

Andreas Kuhn has worked with RNA for almost thirty years. This started with his diploma and PhD theses on the structure and function of small non-coding RNAs using biochemical and molecular biology methods. In his post-doctoral work, Andreas studied RNA-protein interactions in the spliceosome in yeast and later worked on small molecules to affect pre-mRNA splicing. His work on mRNA-based immunotherapies began in 2007 in the academic group of Ugur Sahin at the University Clinic Mainz, and Andreas joined BioNTech SE shortly after its founding in 2008. In his current role as Senior Vice President RNA Biochemistry & Manufacturing the main focus is expanding proprietary technologies to increase the efficacy of mRNA-based therapies and to develop and optimize GMP-compatible manufacturing processes and analytical methods for RNA. He has co-authored numerous publications and patents ranging from basic research on RNA to its application as a therapeutic agent and vaccine.



Marcel Hoefnagel

Medicines Evaluation Board (MEB), NL

Biologist, with a PhD in (Plant) Biochemistry). Assessor of Biopharmaceuticals, specialised in vaccines, allergens, biosimilars, immunogenicity, and cell and gene therapy products. Alternate in Biologics Working Party (BWP). Since January 2023 Chairman of the Quality Innovation Group (QIG; EMA). The QIG supports the translation of innovative approaches to the design, manufacture and quality control of medicines. Since 2020 Chairman of the Substance Validation Group (SVG). The SVG is involved in development and maintenance of EU-SRS (EU- Substance Registration System). Involved in various research projects to support the assessment of (bio)pharmaceuticals and other regulatory activities.

Session 3 - Application of RNA technologies: Non-Clinical



Brigitte Anliker

Paul Ehrlich Institute (PEI), DE

Brigitte Anliker is a scientific assessor for Gene Therapy Medicinal Products (GTMPs) at the Paul-Ehrlich Institute in Germany. She did her Ph.D. thesis in the field of neurobiology at the Max-Planck Institute for Brain Research in Frankfurt, Germany. Thereafter, she was a postdoctoral researcher at the Scripps Research Institute in La Jolla, USA, and at the Heinrich-Heine University in Düsseldorf, Germany. Brigitte joined the Paul-Ehrlich Institute in 2008, where she has been working for 15 years as a non-clinical assessor evaluating clinical trials and marketing authorization applications of GTMPs. Since 2022 she is also member of the Non-clinical Working Party at the European Medicines Agency.



Haiyan Zhou

University College London (UCL), UK

Dr Haiyan Zhou is an Associate Professor in the Genetics & Genomic Medicine Department at University College London (UCL). Her research focuses on preclinical development of RNA-targeted novel therapies and translational biomedical research in rare genetic disorders. Dr Zhou is the deputy lead of the Novel Therapy Theme in the NIHR Biomedical Research Centre at UCL Great Ormond Street Hospital, and leading the RNA therapy Programme at the Great Ormond Street Institute of Child Health. She is also the UK representative and management committee member of the European Cooperation in Science and Technology (e-COST) on 'Delivery of Antisense RNA Therapeutics'.



Susan Goody

Moderna, US

Susan Goody obtained a PhD in Neuroscience from the University of Colorado Health Science Center. Following a post-doctoral fellowship at Yale University, Susan worked at Pfizer, where she worked within Drug Safety Research and Development and developed an expertise in safety pharmacology, non-clinical safety evaluations and regulatory submission strategies to support both small molecules and biotherapeutics. In 2020, Susan was recruited to Moderna, where she leads a team that develops and executes non-clinical safety strategies to support clinical development of mRNA-based therapeutic drug candidates across a diverse therapeutic and vaccine portfolio. Her team is responsible for delivering all non-clinical safety modules of regulatory dossiers and interactions with global health authorities from pre-IND/CTA to post-marketing.



Camilla Svensson

Medical Products Agency (MPA), SE

Camilla Svensson obtained a PhD within the field of immunotoxicology at Uppsala University. Following a post-doctoral period at the Department of Immunology, University of Toronto, she was recruited to the Swedish Medical Products Agency where she has been working for 15 years as a nonclinical assessor at the Unit for Clinical Trials gaining specific expertise in biologics including advanced therapies and oligonucleotide-based therapies. She now holds the position as Scientific Director in Pharmacology and Toxicology at MPA. Camilla is also a member of the Joint 3Rs Working Party at the European Medicines Agency.



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Session 4 - Application of RNA technologies: Clinical



Ralf Herold

European Medicines Agency (EMA)

Ralf Herold is Senior scientific officer in the Regulatory Science & Innovation Task Force (TRS) responsible for Academia liaison, Horizon scanning, Regulatory science collaborations. He is a medical doctor specialised in paediatric and adolescent medicine, experienced in appraising drug development programs, with special interests in data and regulatory science research.



David Henshall

Royal College of Surgeons (RCSI), IE

David is a professor in the department of physiology & medical physics at the RCSI University of Medicine & Health Sciences in Dublin, Ireland. David earned his BSc. in pharmacology from the University of Bristol and PhD in neuropharmacology at the University of Edinburgh. He worked in the USA as a postdoc and then started his laboratory in Ireland in 2004. David's research focuses on cell and molecular mechanisms of epilepsy and their translation to diagnostics and treatments, particularly the therapeutic targeting of RNAs. He is the current Chair of the ILAE's Neurobiology Commission and Director of the FutureNeuro SFI Research Centre for Chronic & Rare Neurological Disorders



Michael Wenger

BioNTech SE, DE

Dr Wenger studied Medicine at Universities of Heidelberg and Freiburg (Germany), and at University of Missouri, Columbia and New York University, New York. He Graduated in 2004. His Doctoral thesis was on radionuclides use in SPECT imaging in cardiovascular patients. He also obtained an Internal Medicine, and Hematology and Oncology Board Certification at the University of Freiburg, Germany under Prof. Roland Mertelsmann. He also spent 10 years at the University of Freiburg 1994-2003), mainly focussing on hematology and oncology as well as working in large tertiary hospitals as. Chief of emergency room. In 2003 he joined Aventis, Germany (now part of Sanofi) as Medical Manager. In 2004 Dr Wenger joined Roche in Basel, Switzerland as International Medical Manager in medical Affairs. From 2012 to 2014, he worked in Genentech (San Francisco, USA). He joined Novartis in June 2019. In 2022 he became Vice President of Clinical Development in BioNTech SE.



Joop van Gerven

Central Committee on Research Involving Human Subjects (CCMO), NL

Joop van Gerven has been chairman of the CCMO since 2016. He is a neurologist and clinical pharmacologist. In addition to being chairman of the CCMO, he is Research Director CNS at the Center for Human Drug Research in Leiden and professor of clinical neuropsychopharmacology at the Leiden University Medical Center. Van Gerven has been a member of the CCMO as a clinical pharmacologist since 2011 and was vice-chairman and chairman ad interim before being appointed chairman.

Session 5 – Panel discussion - Additional panellists



Anna Maria Gerdina Pasmooij

Medicines Evaluation Board (MEB), NL

Marjon Pasmooij, is head of the Science Department at the Dutch Medicines Evaluation Board (MEB) and co-chair of the Regulatory Science Network Netherlands (RSNN). Marjon studied Cell Biology in Wageningen, and did her PhD at the Dermatology Department of the University Medical Center Groningen (UMCG) on a rare genetic skin disease epidermolysis bullosa (EB). Marjon has been employed by the MEB in various roles since 2007. She worked as a clinical assessor for 10 years in the field of gynaecology and dermatology. During these years she combined her regulatory position with a principal investigator position at the UMCG, where she investigated antisense oligonucleotides as a treatment for EB. Since 2018 she is heading the Science Department, and coordinating the Regulatory Science activities where the MEB is involved in (about 15 PhD projects, and 20-25 Master students yearly). She is also part of the EMA-HMA EU-Innovation Network, which facilitates the development of innovative medicines and associated technologies and aims to strengthen engagement with innovators, and a member of the EMA-HMA Big Data Steering Group that is working on the 10 priority recommendations regarding Big Data.



Arjon Van Hengel

DG Research and Innovation, EC

Arjon van Hengel works for the European Commission where he is currently the deputy Head of Unit in the Health Innovations & Ecosystems Unit within the Directorate-General for Research and Innovation. This unit aims to foster the development and uptake of breakthrough innovations in the field of health and care, and to help citizens stay healthy in particular through health promoting environments and people-centred healthcare systems. Arjon has been at the European Commission since 2005 and has worked on various aspects of research related to human health. He has been team leader for infectious diseases during the first one and a half years of the COVID-19 pandemic, policy officer for antimicrobial resistance research and has led a research group that develops and validates analytical detection methods for food allergies. Prior to joining the European Commission, he was a research scientist at the John Innes Centre (Norwich, UK). Arjon studied biology at the University of Utrecht (NL) and received a PhD in molecular biology from the University of Wageningen (NL).



Mariette Driessens

Patient Alliance for Rare Diseases (VSOP), NL

Mariette Driessens is policy officer at VSOP - Patient Alliance for Rare and Genetic Diseases and a volunteer for the haemophilia patient society NVHP, in the Netherlands. As former patient representative of the Committee for Advanced Therapies, she is stimulating patient engagement in application of innovative genetic therapies in the clinic. With several patient organisations, VSOP founded the Patient Platform Gene & Cell therapy in the Netherlands thus sharing knowledge to improve collaboration for access and (inter)national connection. Mariette is currently a member of the ATMP expert group of the Dutch Medicines Evaluation Board, and the Round table on Orphan Drugs at the Dutch HTA agency (Zorginstituut).



Jeske Smink

Silence Therapeutics GmbH, DE

Jeske Smink is a biologist with over 20 years of experience in the biotech industry and academic research. She has expertise in CMC aspects in different areas, including cell therapies, biologics, and siRNAs. After 7 years in academic research focusing on molecular mechanisms in bone development and diseases, she moved to co.don AG as Head of Scientific Affairs in 2012. Here her work focused on preclinical development and quality aspects of cartilage cell transplants, being quality expert for the EMA central market authorization of this cell therapy. Following, she joined ProBioGen AG as CMC project leader, where she was overseeing customer projects, focusing on process development, scale-up and large scale GMP manufacturing of biologics. In 2020, Jeske joined Silence Therapeutics as Head of Drug Substance overseeing CMC development, including process and analytical development, scale-up and clinical product manufacturing of siRNA compounds.

