Regulatory and scientific virtual conference on RNA-based medicines

2 February 2023, 09:00 – 16:30 (CET)

Background and objectives

The European Medicines Agency (EMA) is convening a virtual conference on 2 February 2023 to promote the development of RNA-based medicines, with the following objectives:

- To identify scientific and regulatory opportunities and challenges of RNA-based innovative medicines;
- To facilitate dialogue between industry/academia and regulators and raise awareness on scientific and regulatory aspects of emerging RNA technologies;
- To identify gaps in regulatory science.

This initiative addresses Goal 1 (Catalysing the integration of science and technology in medicines development) of the EMA Regulatory Science Strategy to 2025.

The conference focuses on emerging RNA technologies beyond vaccines.

RNA technologies for gene editing and primary prevention of infectious diseases is not within the scope of this event.

Regulatory and scientific virtual conference on RNA-based medicines

Chaired by Steffen Thirstrup (EMA) and Sol Ruiz (AEMPS)

Welcome and Introduction

09:00 – 09:30	**Joining and technical checks**	**30'**
09:30– 09:45	**Welcome address and opening remarks** *Emer Cooke (EMA)* **Conference goals and anticipated outcome** *Steffen Thirstrup (EMA)*	**15'**

Session 1: State of the art of RNA technologies

Moderated by Falk Ehmann (EMA)

09:45 – 10:30	**Emerging trends on RNA technologies and synthetic oligonucleotide** ***Regulators' perspective:*** *Sol Ruiz, Agency of Medicines and Medical Products (AEMPS), ES* ***Industry perspective:*** *Tal Zaks, OrbiMed, US* ***Academic perspective:*** *Annemieke Aartsma-Rus, Leiden University Medical Center, NL*	**45'**
10:30 – 10:45	**Q&A**	**15'**
10:45 – 11:00	**Coffee break**	**15'**

Session 2: Application of RNA technologies: CMC

Moderated by Brian Dooley (EMA)

11:00 – 12:00	***Opportunities and challenges***	**60'**
	EU Regulators' experience with synthetic oligonucleotides and mRNA technology	
	René Thürmer, Federal Institute for Drugs and Medical Devices (BfArM), DE	
	Industry perspective on synthetic oligonucleotides	
	Daniel Capaldi, Ionis Pharmaceuticals, Inc., US	
	Industry perspective on mRNA technology	
	Pawel Widomski, BioNTech SE, DE	
12:00 – 12:30	**Discussion and Q&A**	**30'**
	Lubomir Nechev, Alnylam Pharmaceuticals, Inc., US	
	Andreas Kuhn, BioNTech SE, DE	
	Marcel Hoefnagel, Medicines Evaluation Board (MEB), NL	
12:30 – 13:30	**Lunch break**	**60'**

Session 3: Application of RNA technologies: Non-Clinical

Moderated by Brigitte Anliker (Paul Ehrlich Institute (PEI), DE)

13:30 – 14:00	**Opportunities and challenges in non-clinical development**	**30'**
	Academic perspective:	
	Haiyan Zhou, University College London (UCL), UK	
	Industry perspective:	
	Susan Goody, Moderna, US	
	Regulators' perspective:	
	Camilla Svensson, Medical Products Agency (MPA), SE	
14:00 – 14:30	**Discussion and Q&A**	**30'**

Session 4: Application of RNA technologies: Clinical

Moderated by Ralf Herold (EMA)

14:30 – 15:00	**Opportunities and challenges in clinical development** ***Academic perspective:*** *David Henshall, Royal College of Surgeons (RCSI), IE* ***Industry perspective:*** *Michael Wenger, BioNTech SE, DE* ***Regulators' perspective:*** *Joop van Gerven, Central Committee on Research Involving Human Subjects (CCMO), NL*	**30'**
15:00 – 15:15	**Discussion and Q&A**	**15'**
15:15 – 15:30	**Coffee break**	**15'**

Session 5: Panel discussion

Moderated by Marjon Pasmooij (Medicines Evaluation Board, MEB) and Steffen Thirstrup (EMA)

15:30 – 16:20	**Panel discussion on how to support development of RNA-based medicines – Multilateral perspectives** ***Chairs and speakers from previous sessions:*** *Sol Ruiz, (AEMPS), Annemieke Aartsma-Rus (LUMC)* *Tal Zaks (OrbiMed), Marcel Hoefnagel (MEB),* *Susan Goody (Moderna), Joop van Gerven (CCMO)* ***Additional panellists:*** *Arjon Van Hengel, DG Research and Innovation, EC* *Mariette Driessens, Patient Alliance for Rare Diseases (VSOP), NL* *Jeske Smink, Silence Therapeutics GmbH, DE*	**50'**
16:20 – 16:30	**Closing remarks** *Sol Ruiz (AEMPS), Steffen Thirstrup (EMA)*	**10'**