EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Orphan Medicines Development – ask the European regulator

Webinar on Rare World Diseases Day

29 February 2024
Virtual meeting via WebEx / EMA, Amsterdam

The EMA's Orphan Medicines Office is hosting an interactive webinar on **Rare Disease Day** on 29 February 2024, to answer questions that developers of products for rare diseases or researchers in this field might have on orphan designations and rare disease development.

The webinar will feature:

- short presentations on the background of orphan designation and the benefits it has brought to patients;
- a live question-and-answer session enabling participants to ask their questions on orphan medicines development to a panel of regulatory experts.

Orphan Medicines Development – ask the European Regulator

16:00	**Welcome**	**5'**
	Melanie Carr *Head of Stakeholders and Communication (EMA)*	
16:05	**Introduction to orphan regulation**	
	Kristina Larsson *Head of Orphan Medicines Office (EMA)*	**10'**
16:15	**Importance of rare disease development and patient engagement**	
	Virginie Hivert *EURORDIS*	**10'**
16:25	**Q & A Session**	
	Pannel of experts on different topics[1] Moderator: **Melanie Carr** *Head of Stakeholders and Communication (EMA)*	**60'**
17:25	**Conclusions**	
	Kristina Larsson *Head of Orphan Medicines Office (EMA)*	**5'**

[1]List of expert panellists

Theodor Framke – EMA, clinical trial methodology

Violeta Stoyanova-Beninska – MEB, Committee for Orphan Medicinal Products (COMP) Chair

Iordanis Gravanis – EMA, scientific advice and protocol assistance

Maria Mavris – EMA, patient engagement

Maribel Rico-Salas –EMA, academia liaison

Hélène Le Borgne – EC, Directorate General for Research and Innovation

About the speakers

Melanie Carr

Head of Stakeholders and Communication, EMA

Melanie Carr is Head of the Stakeholders and Communication Division and a member of the Executive Board at the European Medicines Agency (EMA). She joined EMA in 1996 and has previously held various roles in pharmacovigilance, the centralised procedure for marketing authorisation, orphan medicines, the SME office and corporate stakeholders department. In her current role she is also responsible for crisis management. She has a degree in Pharmacy from the University of Nottingham in the UK and worked as a regulatory professional in the pharmaceutical industry prior to joining EMA.

Kristina Larsson

Head of Orphan Medicines Office, EMA

Kristina Larsson is the Head of the Orphan Office at EMA and the scientific lead of the Committee of Orphan Medicinal Products (COMP), a position she has held since July 2014. Prior to this she spent 8 years as a scientific officer in the scientific advice team of the EMA, mostly focusing on oncology, inborn errors of metabolism and biosimilar monoclonal antibodies. Before joining the Agency she worked three years in clinical research for AstraZeneca in Mölndal, Sweden. Kristina has a master of Medicine in Pharmaceutical Bioscience from the University of Gothenburg.

Virginie Hivert

Therapeutic Development Director, EURORDIS

Joined EURORDIS in June 2014. Between June 2014 and January 2022, she has served as Observer on the EMA Committee for Orphan Medicinal Products (COMP), supportng patient representatives. During this period, she was also chairing the Therapeutic Action Group (TAG) put in place by EURORDIS to give a platform for RD patient representatives who are members of the EMA Scientific Committees. From March 2019 to February 2022, she has been PRAC alternate member representing patient organisations. Since June 2022, she has been appointed as one of the Civil Society representatives on the EMA Management Board. At global level, she is involved in the International Rare Diseases Research Consortium (IRDiRC) since its inception in 2011.