

Optimising Regulatory Strategies for Orphan Drugs

10 November 2026



Programme

Time	Activity	Speakers
08:30	Welcome from TOPRA	TOPRA
08:35	Introductions	
09:05	Orphan Medicinal Product Legislation - Overview of the Frameworks in the EU (UK), US and Japan - What the regulations cover and why, what they try to protect from (i.e. creation of false sub-populations of a non-orphan condition) - Awards for obtaining ODD - Considerations for Orphan Drug Designation - Sequence of submissions by country - Developing orphan versus non-orphan indications - Paediatric conditions including the challenges and impacts in this area, trade-off of the incentives and the ongoing evaluation of the orphan regulation by the EC	Evgenia Mengou <i>EV Pharma Solutions</i>
10:15	Break	
10:20	Obtaining Orphan Drug Designation - Orphan Drug Designation in the EU - Application - Procedure - Similarities and differences with the US - Application, Procedure and Incentives - Rare diseases: a global issue - Collaboration between Agencies - Strategic considerations on when to apply and to what Agencies - ODD in Australia – similarities and differences; application procedure and incentives	Akiko Tagawa <i>Roche Products Ltd</i> Jennifer Svec <i>The Reg Group Pty Ltd</i>
11:25	Case study <i>Participants must read the pre-course material before this session.</i>	Joanna Allen <i>Biogen</i>
12:05	Break	
12:10	Maintenance of Orphan Drug Designation - What and when prior to MAA/NDA - Policy 43 – what it is and its impact - What and when during an MAA/NDA, experiences with OMAR - Assessment of similarity and significant benefit	Adriaan Fruijtier <i>CATS Consultants GmbH</i>
13:10	Lunch	
13:45	EU revision of the Orphan Drugs Legislation	Adriaan Fruijtier

14:30 Orphan Drug Framework around the World

João Duarte
Ipsen

15:10 Q&A

15:25 Closing remarks and feedback

15:45 Close
