

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> Neama Baho <i>Regulatory and resourcing</i>
11:25	Case study <i>Participants must read the pre-course material before this session.</i>	Joanna Allen <i>AstraZeneca</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
