Optimising Regulatory Strategies for Orphan Drugs

30 October 2025

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
		EV Pharma Solutions

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
		Roche Products Ltd
		
		Jennifer Svec
		The Reg Group Pty Ltd

11:25	**Case study** *Participants must read the pre-course material before this session.*	**Joanna Allen** *Biogen*
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
		CATS Consultants GmbH

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**	