Optimising Regulatory Strategies for Orphan Drugs

Online
10 November 2026

Programme

Time	Activity	Speakers
08:45	**Welcome from TOPRA**	*TOPRA*
08:50	**Introductions**	**Adriaan Fruijtier** *CATS Consultants GmbH*
09:00	**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*
10:10	**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* **Neama Baho** *Regulatory and resourcing*
11:25	**Orphan Drug Framework around the World**	**João Duarte** *Ipsen*
12:10	**Case study** *Participants must read the pre-course material before this session.*	**Joanna Allen** *AstraZeneca*
13:00	**Lunch**	
13:45	**Maintenance of Orphan Drug Designation** • What and when prior to MAA/NDA	**Adriaan Fruijtier**

	○ Policy 43 – what it is and its impact • What and when during an MAA/NDA, experiences with OMAR ○ Assessment of similarity and significant benefit	*CATS Consultants GmbH*
14:45	**EU revision of the Orphan Drugs Legislation**	**Adriaan Fruijtier**
15:30	**Q&A**	
15:50	**Closing remarks and feedback**	
16:00	**Close**	