Optimising Regulatory Strategies for Orphan Drugs

Programme

Time	Activity
09:15	Registration
09:30	Welcome and Introduction
09:35	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 o Sequence of submissions by country o Developing orphan versus non-orphan indications o 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
10:35	**Tea/ coffee break**
11:05	Obtaining Orphan Drug Designation • Orphan Drug Designation in the EU o Application o Procedure • Similarities and differences with the US o Application, Procedure and Incentives • Rare diseases: a global issue o Collaboration between Agencies • Strategic considerations on when to apply and to what Agencies • Case study
13:05	**Lunch** *Optional session: An overview of the IRIS system*
14:05	Maintenance of Orphan Drug Designation • What and when prior to MAA/NDA o Policy 43 – what it is and its impact • What and when during an MAA/NDA, experiences with OMAR o Assessment of similarity and significant benefit o Case studies on recent notices (e.g. BMS case) • Case Study
15:05	**Tea/ Coffee break**
15:35	Orphan Drug Designation • A global perspective o Russia, China, Switzerland o Australia
16:35	**Close**