

Optimising Regulatory Strategies for Orphan Drugs

Tuesday 9 April 2019



Programme

Time	Activity	Speaker
09:15	Registration	
09:30	Welcome and Introduction	Samantha Alsbury TOPRA
09:35	Orphan Medicinal Product Legislation <ul style="list-style-type: none">• 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<ul style="list-style-type: none">○ 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	Tara Hutton Biogen Idec
10:35	Tea/ coffee break	
11:05	Obtaining Orphan Drug Designation <ul style="list-style-type: none">• Orphan Drug Designation in the EU<ul style="list-style-type: none">○ Application○ Procedure• Similarities and differences with the US<ul style="list-style-type: none">○ Application, Procedure and Incentives• Rare diseases: a global issue<ul style="list-style-type: none">○ Collaboration between Agencies• Strategic considerations on when to apply and to what Agencies• Case study	Emma Blasi VCN Biosciences S.L. Gillian Lennard Independent Consultant
13:05	Lunch <i>Optional session: An overview of the IRIS system</i>	Tara Hutton Biogen Idec
14:05	Maintenance of Orphan Drug Designation <ul style="list-style-type: none">• What and when prior to MAA/NDA<ul style="list-style-type: none">○ Policy 43 – what it is and its impact• What and when during an MAA/NDA, experiences with OMAR<ul style="list-style-type: none">○ Assessment of similarity and significant benefit○ Case studies on recent notices (e.g. BMS case)• Case Study	Adriaan Fruijtier CATS Consultants GmbH Gillian Lennard DLRC Ltd.
15:05	Tea/ Coffee break	
15:35	Orphan Drug Designation <ul style="list-style-type: none">• A global perspective<ul style="list-style-type: none">○ Russia, China, Switzerland○ Australia	João Duarte Alexion Pharmaceuticals Jennifer Svec The Reg Group Pty Ltd
16:35	Close	