IPI Academy

MANAGEMENT forum

Presented by
Management Forum

Regulatory Strategies for Orphan Drugs

24 June 2024
+ 8 October 2024

This must-attend event will provide an essential overview of the opportunities and challenges presented by orphan drug regulation.

Format:
Live online

CPD:
6 hours for your records

Certificate of completion

Overview

The market for orphan drug products continues to expand and developing new treatments for rare diseases is becoming an increasingly important growth area for many pharmaceutical companies. This interest in rare and orphan diseases is linked not only to the incentives offered by the different regulatory authorities but also to the recent development of advanced therapies, which are often developed for the treatment of rare diseases.

The interactive course will cover all the key aspects of orphan drug legislation in both the US and EU, including a review of changes proposed in the revised EU orphan drug legislation, and the regulatory processes designed to expidite development and thereby enable earlier access to innovative medicines. The programme will explain in detail the key concepts critical for a thorough understanding of the orphan legislation in the EU and US and how it is applied. The two-stage process of first obtaining the orphan designation and then submitting the MAA/NDA and maintaining the designation as an orphan drug in order to benefit from incentives will be described. We will also cover clinical development considerations and flexibilities that are relevant for orphan drugs.

Case studies will consolidate and illustrate the learning points with real world examples and ensure that you leave the event with practical skills and knowledge that can be put to use in the workplace. The course will allow you not only to learn from our expert trainer but also to share the experiences of other delegates.

Benefits of attending

- **Gain** a detailed overview of the orphan medicinal product legislation in the EU and the US
- **Receive** practical advice on how to prepare and manage orphan submissions
- **Assess** strategic considerations of how orphan status can influence drug development programmes
- **Observe** how the theory is applied to real world examples

Who should attend?

This event will be particularly relevant to regulatory affairs professionals who are involved in orphan drug submissions. It will also be of interest to those working in rare diseases who would benefit from an overview of orphan drug designation.

Programme

Orphan Drug Legislation - background and overview

- Origin and history of orphan drug legislation
- EU and US legislation overview and comparison
  - Brief discussion of other countries/regions
- Key concepts and definitions
- Orphan incentives
- Overlaps with paediatric legislation
- FDA and EMA databases

Challenges with orphan definitions and application of the legislation

- Condition vs indication
- "salami-slicing" of indications
- Similarity and sameness
  - Extra challenges of ATMPs
  - Case law
- Significant benefit and superiority
- Market exclusivity

Procedural aspects for orphan designated products

- The two-stage process
  - Orphan designation process EU and US
  - MAA/NDA process for orphan drugs EU and US including maintenance of orphan designation

Case Study 1

- EU orphan drug process

Development considerations for orphan drugs

- Clinical development requirements and flexibilities applied to orphan drugs
- EMA and FDA guidance documents on clinical development relevant to orphan drugs
- Applicability of expedited and early access programmes in EU and US
- New clinical indications
  - Orphan/non-orphan

Case Study 2

- US process and clinical development considerations

Anticipated future developments and changes to the regulation of orphan drugs

- EU and US

Presenter

Shaun Stapleton

Shaun Stapleton is an independent consultant providing regulatory strategy advice and support to biopharma clients globally.

Shaun held positions of increasing responsibility in regulatory affairs at Sterling Winthrop, Eli Lilly, Boehringer Ingelheim and Ipsen, where he managed regulatory input into development programmes globally, securing new product approvals in the neurology, endocrinology and oncology therapeutic areas.

More recently, Shaun spent 8 years in regulatory consultancy at RRG (a Voisin Consulting Life Sciences Company) where he was a Director and Vice President of Regulatory Science working with global clients on a wide range of regulatory projects. From VCLS he moved to ReNeuron Ltd where he led regulatory, pharmacovigilance and latterly quality aspects of cell therapy and exosome development programmes.

Shaun has extensive experience in orphan drug development from first in man clinical trials through to commercialisation globally. He has been involved in discussions with regulators relating to specific orphan exclusivity issues and chaired the Alliance for Regenerative Medicine working group on “sameness” in the context of market exclusivity for orphan ATMPs (cell therapy). Most recently he was VP and Head of Global Regulatory Affairs at Amryt Pharma, a company specialising in the development and commercialisation of orphan medicines globally, where he contributed to the approval and life cycle management of several orphan drugs in areas of high unmet medical need.

Course dates

24 June 2024	**Live online** 09:30-17:00 **UK (London)** (UTC+01) *Course code 13737*	GBP **549** ~~649~~ EUR **789** ~~929~~ USD **893** ~~1,049~~ **Until 20 May**
8 October 2024	**Live online** 09:30-17:00 **UK (London)** (UTC+01) *Course code 13919*	GBP **549** ~~649~~ EUR **789** ~~929~~ USD **893** ~~1,049~~ **Until 03 Sep**

How to book

Online:
ipi.academy/2455

Alternatively contact us to book, or if you have any queries:

Email:
info@ipi.academy

Phone:
+44 (0)20 7749 4749

Discounts

- Booking more than one delegate on any one date qualifies for a **15% discount** on the second and subsequent places.
- Most events qualify for an **early booking discount** prior to 6 weeks before the course date. Be sure to check on our website, where the latest discounts will be shown.

Further information

Fee
The fee includes all meals and refreshments for the duration of the course (for venue-based courses) and a complete set of course materials (provided electronically). If you have any particular requirements, please advise customer services when booking.

Please note
IPI Academy (and our training partners) reserve the right to change the content and timing of the programme, the speakers, the date and venue due to reasons beyond their control. In the unlikely event that the course is cancelled, we will refund the registration fee and disclaim any further liability.

Terms and conditions
The rest of the our terms, the event cancellation policy and the terms and conditions are on our website, please visit ipi.academy/content/terms-and-conditions

Reviews

★★★★★

I believe as a starting point into the world of ODD this was very comprehensive and informative. Very clear and concise.

Khayyam Darr
Senior Regulatory Affairs Executive
Chiesi Ltd
Sep 26 2022

★★★★★

Superb, excellent delivery and very knowledgeable [...] the best training course I attended in a log time!

John McIntyre
Senior Consultant
Parexel Ireland USD
Jun 20 2022

★★★★★

Excellent would attend other similar webinar/trainings

Cheryl Dicks
Sr. Mgr. Regulatory
CANbridge Pharmaceuticals
Mar 16 2022

★★★★☆

Very interactive session (nicely done even if remote) Expertise sharing and specific examples shared during the training to emphasize and help to understand key topics

Christine Monteiro
Senior Regulatory Project Manager
IPSEN
Sep 26 2022

Run this programme in-house for your whole team

Coming to IPI Academy for your in-house training provides an all-inclusive service which gives you access to a wide variety of content, learning platforms and delivery mechanisms as well as your own personal training adviser who will work with you from the initial enquiry through to feedback and follow-up after the programme.

With over 600 trainers, all practitioners and experts across a huge range of fields, we can provide the training you need, where you need it, when you need it, and at a price which suits your budget. Our approach to tailored learning and development consists of designing and delivering the appropriate solution for each client.

For your FREE consultation and to find out more about how we can work with you to solve your training needs, please contact our training advisers:

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IPI Academy

IPI Academy is a training initiative of Falconbury and Management Forum; leading providers of industry training for over 30 years, based in the UK.

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