IPI Academy

MANAGEMENT forum

Presented by
Management Forum

Orphan Drug Regulatory Masterclass: EU & US Insights

14 October 2025

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 orphan drug market is rapidly expanding, with the development of new treatments for rare diseases becoming a key growth area for many pharmaceutical companies. This rising interest in rare and orphan conditions is fuelled not only by attractive incentives from regulatory authorities but also by the emergence of advanced therapies, many of which target rare diseases.

This highly interactive course provides comprehensive coverage of orphan drug legislation in both the EU and US, including an overview of changes to the EU orphan drug framework being proposed as part of the ongoing comprehensive review of the EU pharmaceutical legislation. You'll gain a clear understanding of the regulatory mechanisms designed to accelerate development and improve early access to innovative treatments.

Through expert-led sessions and detailed guidance, the course explores the core concepts essential to navigating orphan drug legislation. It walks you through the entire process - from obtaining orphan designation during development through to maintaining the designation to secure key incentives following submission and approval of the MAA/NDA.

We'll also examine clinical development flexibilities specific to orphan drugs, offering insight into the unique regulatory considerations and strategies that support innovation in this space.

Real-world case studies bring the concepts to life, consolidating your learning and ensuring you leave with practical knowledge and actionable insights you can apply immediately. Alongside our experienced trainer, you'll also benefit from connecting with other professionals in the field and sharing valuable perspectives.

Benefits of attending

- **Gain** a detailed overview of orphan medicinal product legislation in the EU and US, equipping you with the essential regulatory knowledge to navigate this evolving landscape with confidence
- **Receive** practical advice on effectively preparing and managing orphan submissions, helping you streamline processes and avoid common pitfalls
- **Assess** strategic considerations for leveraging orphan status to shape and optimise drug development programmes
- **Deepen** your understanding through real-world examples and experience-based insights that bring the theory to life

Who should attend?

This programme is especially valuable for:

- Regulatory affairs professionals involved in orphan drug submissions - offering targeted insights to support your work in this highly specialised area
- Professionals working in the field of rare diseases - providing a clear and comprehensive overview of orphan drug designation and its practical implications for drug development

Programme

Orphan Medicinal Product Legislation

- Overview of the legislation in the EU, US and Japan
  - Including discussion of the ongoing revision to the EU legislation
- What the Regulations cover and why, what they try to protect
- Benefits of Orphan Drug Designation
- Key Considerations
  - Country Submissions
  - Differences of Orphan versus non-orphan
  - Paediatric Conditions

Obtaining Orphan Drug Designation

- Orphan drug designation in the EU
  - Application
  - Procedure
- Similarities and differences with the US
  - Application, procedure and incentives
- Global Issues around Rare Disease
- Strategic Considerations
- Case Study

Maintenance of Orphan Drug Designation

- How to maintain
- Maintenance during MAA and NDA
- Assessment and Case Study

Global Environment

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 date

14 October 2025

Live online
09:00-17:00 **UK (London)** (UTC+01)
Course code 14982

GBP **649** ~~749~~
EUR **909** ~~1,049~~
USD **1,043** ~~1,199~~
Until 09 Sep

How to book

Online:
ipi.academy/2455

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

Email:
info@ipiacademy.com

Phone:
+44 (0)20 7749 4749

Discounts

- Booking more than one delegate on any one date qualifies for a **30% 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

★★★★★

Excellent training.

Thiago Loureiro
Sr. manager , Regulatory Strategy
Vertex
Jun 24 2024

★★★★★

Very instructive webinar. Although packed, the day went by quickly.

Ariane Teyssou
Regulatory Affairs Specialist
Enterome
Jun 24 2024

★★★★☆

Well organised and very experienced presenter

Maria Liljevald
Head of Global Regulatory Affairs
Ribocure Pharmaceuticals AB
Jun 24 2024

★★★★★

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

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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