Advanced Regulatory Strategy for Orphan Drugs Training Course

Professional Development Training Course Outline

Category	Biotechnology and Pharmaceutical Development	**Duration**	10 Days
Base Fee	$4,000 USD	**Delivery Mode**	Online / On-site Hybrid

Course Introduction

The orphan drug landscape is rapidly evolving, driven by breakthroughs in precision medicine, cell and gene therapy (CGT), and a global commitment to addressing unmet medical needs in rare diseases. Since the enactment of the Orphan Drug Act, the regulatory environment has become increasingly complex, demanding specialized expertise to navigate the high-stakes pathways in the US, EU, and emerging markets. Success requires an integrated regulatory strategy that maximizes incentives such as marketing exclusivity and user fee waivers while adapting to flexible yet rigorous clinical trial methodologies in small patient populations. Advanced Regulatory Strategy for Orphan Drugs Training Course is designed to move beyond the basics of designation, focusing instead on lifecycle management, global harmonization (ICH), and strategic commercialization planning from pre-clinical through post-approval.

This intensive course will equip regulatory affairs, clinical development, and portfolio management professionals with the next-generation regulatory intelligence needed to accelerate development timelines and secure market access for innovative therapies. We will dissect the current EU pharmaceutical legislation reform, the strategic implications of pediatric investigation plans (PIPs), and the critical importance of a clinically superior product to maintain orphan exclusivity. Through advanced case studies and interactive workshops, participants will master risk-based regulatory decision-making, optimize their health technology assessment (HTA) preparation, and formulate robust, global regulatory roadmaps that translate scientific promise into life-saving treatments for patients with rare conditions.

Programme Curriculum

Advanced Regulatory Strategy for Orphan Drugs Training Course

Introduction

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Course Objectives

Upon completion, participants will be able to:

1. Evaluate the impact of the pending EU Pharma Legislation Reform on the Orphan Medicinal Product (OMP) framework.
2. Formulate a successful Orphan Drug Designation (ODD) strategy across US FDA (OOPD) and EU EMA (COMP), including justification for prevalence and significant benefit.
3. Design adaptive clinical trials and leverage master protocols and Bayesian methods to overcome challenges in small patient populations.
4. Integrate real-world evidence (RWE) and digital health technologies (DHTs) into regulatory submissions for enhanced product demonstration.
5. Strategize for the achievement and defense of 7-year US and 10-year EU Marketing Exclusivity and the Clinical Superiority requirement.
6. Develop robust Pediatric Investigation Plans (PIPs) or Waiver Justifications to ensure timely EU submission clearance.
7. Manage the regulatory lifecycle of Cell & Gene Therapies (CGT) and other Advanced Therapy Medicinal Products (ATMPs) with Orphan status.
8. Conduct strategic pre-submission meetings and Protocol Assistance with key global agencies
9. Translate regulatory data into successful Health Technology Assessment (HTA) dossiers for early access and pricing/reimbursement.
10. Mitigate regulatory risk associated with rare disease diagnostics and combination products.
11. Master the process of ODD maintenance and its critical link to final MAA/NDA approval.
12. Define and implement a harmonized eCTD submission strategy for global registration.
13. Apply ethical and regulatory guidelines to the use of AI/ML in rare disease drug development.

Target Audience

1. Regulatory Affairs Professionals
2. Clinical Development/Operations Leads focusing on rare diseases
3. Project/Portfolio Managers overseeing Orphan Drug pipelines
4. R&D Scientists transitioning into translational and clinical stages
5. Biotechnology/Start-up Executives seeking to leverage ODD incentives
6. Medical Affairs/Medical Writers supporting ODD applications and clinical dossiers
7. Legal/Compliance professionals specializing in pharmaceutical exclusivity and IP
8. Commercial/Market Access teams preparing for HTA and reimbursement negotiations

Course Modules

Module 1: Global Foundations of Orphan Drug Legislation

- In-depth review of the US Orphan Drug Act (ODA) and EU Regulation (EC) No 141/2000.
- Defining a rare disease and substantiating prevalence and significant benefit criteria.
- Analyzing the financial and market advantages
- Understanding the functions of FDA's OOPD and EMA's COMP.
- Case Study: Dissecting a successful US ODD submission for an ultra-rare metabolic disorder with challenging prevalence data.

Module 2: Strategic Orphan Drug Designation (ODD) Filing

- Optimal timing for ODD application relative to IND/CTA filing.
- Preparing the non-clinical, clinical, and scientific rationale for designation.
- Navigating ODD submissions when a "similar product" exists.
- Strategies for concurrent US and EU designation applications.
- Case Study: EMA COMP rejection of ODD based on insufficient significant benefit claim over a decades-old standard-of-care.

Module 3: Advanced Clinical Trial Design for Rare Diseases

- Utilizing master protocols, umbrella, basket, and platform trials.
- Leveraging Bayesian statistics and other methods for small sample sizes.
- Justification of clinically meaningful and surrogate endpoints in rare conditions.
- Integrating patient advocacy and PROs into trial design.
- Case Study: The use of an adaptive design and natural history study to achieve approval for a Duchenne Muscular Dystrophy (DMD) therapy.

Module 4: Global Regulatory Pathways and Expedited Review

- Utilizing Fast Track, Breakthrough Therapy, Accelerated Approval, and Priority Review.
- Leveraging the PRIME scheme.
- Strategies for utilizing a rolling submission process to expedite review time.
- Optimal planning and content for FDA Type B and EMA Scientific Advice/Protocol Assistance meetings.
- Case Study: A sponsor's successful use of the FDA Breakthrough Therapy designation to bypass a full-length Phase III trial.

Module 5: Defending and Maintaining Orphan Marketing Exclusivity

- Exclusivity Rules.

- Providing evidence of significant advantage to overcome a competitor's exclusivity.
- Understanding the pitfalls, including failure to meet supply demand or regulatory inaccuracies.
- Strategy for seeking multiple ODDs for different rare sub-indications of the same drug.
- Case Study: The regulatory challenge and resolution of a US exclusivity dispute involving a second-in-class orphan biologic.

Module 6: Pediatric Strategy and the PIP/PSP

- Mandatory requirements, deadlines, and waiver/deferral applications.
- Alignment with PIPs and compliance with PREA
- Incentive Mechanisms.
- Challenges in selecting and testing pediatric formulations for rare diseases.
- Case Study: A successful PIP deferral strategy that aligned with the delayed adult clinical trial timeline for a rare pediatric cancer drug.

Module 7: Advanced Therapies (ATMPs) and Orphan Status

- The intersection of Cell and Gene Therapy regulatory pathways with Orphan designation.
- Chemistry, Manufacturing, and Controls unique to ATMPs for rare diseases.
- Regulatory considerations for ATMP delivery to small, dispersed patient groups.
- Applying quality risk management to novel therapies.
- Case Study: A Gene Therapy's expedited approval based on EMA ATMP Committee review and its subsequent ODD maintenance.

Module 8: Global Regulatory Roadmap and Lifecycle Management

- Determining the optimal FDA, EMA, PMDA submission order.
- Best practices for compiling and submitting a global electronic Common Technical Document (eCTD).
- Strategic variations, amendments, and annual report submissions.
- Harmonizing global labeling, especially the "Orphan Indication" text.
- Case Study: A pharmaceutical company's eCTD submission strategy for concurrent US and EU approval of a new chemical entity (NCE) for an Orphan indication.

Module 9: Real-World Evidence (RWE) & Digital Health

- Using Real-World Evidence to support natural history and post-approval commitments.
- Agency perspectives on RWE for supplementing or replacing traditional control data.
- Utilizing Digital Health Technologies for remote monitoring in rare disease trials.
- Exploring innovative data submission models with regulators.
- Case Study: Approval of a drug using a patient registry as the primary source of RWE to support a post-marketing requirement.

Module 10: Regulatory and Commercialization Interface

- Aligning the regulatory dossier to anticipate Health Technology Assessment requirements.
- Understanding the impact of ODD exclusivity on market access strategy.
- Navigating compassionate use, expanded access, and Named Patient Programs.
- Assessing the risk of ODD revocation post-approval based on market size.
- Case Study: A company's pre-emptive HTA submission for a high-cost orphan drug and its successful negotiation for reimbursement in a key EU market.

Module 11: Manufacturing and Quality (CMC) for Orphan Drugs

- CMC documentation tailored for the unique challenges of small batch sizes.
- eCTD Module 3 content and the Quality Overall Summary (QOS) for novel rare disease products.
- Supply Chain Resilience.
- Lifecycle management and post-approval change protocols for complex biologics.
- Case Study: Overcoming an FDA Complete Response Letter (CRL) due to deficiencies in manufacturing controls for a sterile orphan injectable.

Module 12: Pharmacovigilance and Post-Marketing Commitments

- Developing focused RMPs appropriate for the rare disease safety profile.
- Challenges of Pharmacovigilance and Signal Detection in ultra-rare patient groups.
- Strategic design and execution of Phase IV confirmatory trials.
- Regulatory implications of a supply disruption for a sole-source orphan drug.
- Case Study: An EU post-marketing commitment to a Phase IV study for long-term safety data in a previously unstudied pediatric subpopulation.

Module 13: Regulatory Ethics and Patient Advocacy

- Ethical and regulatory role of the patient in clinical trial design and regulatory review.
- Challenges in obtaining truly informed consent in vulnerable rare disease populations.
- Addressing specialized ethical concerns related to novel therapies and small patient cohorts.
- Strategic collaboration with PAGs to enhance trial recruitment and regulatory messaging.
- Case Study: A protocol amendment stemming from patient group feedback regarding the frequency of invasive procedures in a long-term rare disease trial.

Module 14: EU Pharmaceutical Legislation Reform Impact

- Detailed review of the key elements of the proposed new EU Pharma Package impacting OMPs.
- Analysis of proposed changes to the 10-year market exclusivity period and conditions.
- The new focus on meeting a High Unmet Medical Need (HUMN) for maximum incentives.
- Steps to transition a pipeline from the current to the future EU framework.
- Case Study: Hypothetical scenario analysis: How an existing ODD would be impacted by the new EU regulations regarding unmet need.

Module 15: Regulatory Futures: AI and Global Convergence

- Ethical and regulatory use of Artificial Intelligence (AI) for data generation and submission efficiency.
- Latest updates from the ICH on harmonized rare disease guidelines.
- Predicting the increasing reliance on RWE for initial and expanded indications.
- Building a highly efficient, digitally-enabled, global regulatory team.
- Case Study: An early-phase trial leveraging AI to rapidly identify and screen rare disease patients across multiple geographies.

Training Methodology

- Interactive Workshops.
- Expert-Led Lectures.
- Real-World Case Studies.
- Q&A/Panel Discussions.

Register as a group from 3 participants for a Discount

Send us an email: info@fineskilltrainingcenter.org or call +254769199797

Certification

Upon successful completion of this training, participants will be issued with a globally- recognized certificate.

Tailor-Made Course

We also offer tailor-made courses based on your needs.

Key Notes

a. The participant must be conversant with English.

b. Upon completion of training the participant will be issued with an Authorized Training Certificate

c. Course duration is flexible and the contents can be modified to fit any number of days.

d. The course fee includes facilitation training materials, 2 coffee breaks, buffet lunch and A Certificate upon successful completion of Training.

e. One-year post-training support Consultation and Coaching provided after the course.

f. Payment should be done at least a week before commence of the training, to Fineskill Training Center account, as indicated in the invoice so as to enable us prepare better for you.

Upcoming Scheduled Sessions

Start Date	End Date	Location	Price
23 Nov 2026	04 Dec 2026	Online	$2,000
23 Nov 2026	04 Dec 2026	Physical	$3,000
23 Nov 2026	04 Dec 2026	Physical	$6,000
23 Nov 2026	04 Dec 2026	Physical	$6,000
23 Nov 2026	04 Dec 2026	Physical	$12,000
23 Nov 2026	04 Dec 2026	Physical	$9,000
23 Nov 2026	04 Dec 2026	Physical	$12,000
23 Nov 2026	04 Dec 2026	Physical	$12,000

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