Orphan Drug Designation

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Developing a treatment for a rare disease?

Every strong orphan drug strategy starts with asking the right questions

01 **ELIGIBILITY**
Is the target condition eligible for ODD?

02 **PREVALENCE**
Is the prevalence of the condition eligible for ODD?

03 **TERRITORY**
Is the prevalence of the condition eligible for ODD?

04 **INCENTIVES**
Which development strategy maximizes incentives in the different territories?

05 **EU REFORM**
How could future changes affect exclusivity?

One global program. Different regulatory frameworks. One coordinated strategy.

Supporting orphan programmes in Europe and the US

Asphalion can coordinate a consistent global strategy while addressing jurisdiction-specific requirements

EUROPEAN UNION | EMA

1. ODD eligibility assessment
2. Orphan designation strategy, including Significant Benefit
3. Submission preparation
4. EMA preparatory interactions
5. Final Submission
6. Life-cycle support

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GLOBAL ODD STRATEGY DURING DEVELOPMENT

From feasibility to life-cycle

UNITED STATES | FDA

1. ODD eligibility assessment
2. Orphan designation strategy
3. Submission preparation and filing
4. Life-cycle support

Orphan Drug Designation in the EU

Purpose

In the EU, orphan designation supports the development of medicines intended to diagnose, prevent or treat rare conditions by providing access to specific regulatory incentives.

It is a key tool to facilitate development and regulatory interaction for products targeting rare diseases.

ODD

Status of Orphan Drug Designations in the EU

Requirements

- Life-threatening or chronically debilitating condition.
- EU prevalence ≤5 in 10,000, or insufficient expected return.
- No available methods/therapies for the target condition or a significant benefit over existing methods/therapies.

Benefits

- Access to protocol assistance for designated orphan medicines.
- 10 years Market exclusivity once the medicine is on the market.
- Fee reductions or exemptions for regulatory procedures.

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New EU Pharmaceutical legislation: What could change?

Targeted changes to designation and a more differentiated market exclusivity framework

1 | ORPHAN DESIGNATION

New framework

- **ODD validity limited to 7 years**, with the possibility of extension.
- New **“Breakthrough Orphan Medicinal Product”** category.

Eligibility criteria

Economic-return criterion would be removed.

Designation would focus on:

- Life-threatening or chronically debilitating conditions.
- Prevalence of ≤5 in 10,000 people in the EU.

2 | MARKET EXCLUSIVITY

Proposed exclusivity periods

4 years	9 years	11 years
Bibliographic data	Standard orphan	Breakthrough orphan

Additional proposed changes

- A Marketing Authorization Application(MAA) for a similar product may be accepted and validated when less than 2 years of the originator’s market exclusivity period remain.
- A 1-year extension for each new orphan condition, up to a maximum of two extensions.

KEY IMPLICATION: **Market exclusivity would no longer be uniform.** Early product classification and indication planning could directly influence the protection period.

Orphan Drug Designation in the US

Purpose

In the United States, ODD is granted by the FDA to a drug or biological product intended for the prevention, diagnosis or treatment of a rare disease or condition.

The program encourages orphan product development through specific regulatory and financial incentives.



ODD

Status of Orphan Drug Designations in the US

Requirements

- Intended to prevent, diagnose or treat a rare disease or condition.
- Affects <200,000 people in the US, or insufficient expected return from US sales.

Benefits

- Tax credits for qualified clinical trials.
- Exemption from FDA user fees.
- Potential 7 years of orphan-drug exclusivity following approval.
- Eligibility for certain FDA orphan-product development grants.

Supporting orphan programmes in the UK

When to apply?
Together with the MAA

ODD

Status of Orphan Drug Designations in the UK

Purpose

In the UK, orphan status is granted by the MHRA to medicinal products intended for the diagnosis, prevention or treatment of life-threatening or chronically debilitating rare conditions.

Requirements

- Life-threatening or chronically debilitating condition.
- UK prevalence ≤5 in 10,000, or insufficient expected return.
- No satisfactory method available, or significant benefit.

Benefits

- Up to 10 years of market exclusivity.
- Potential 2-year paediatric extension.
- Marketing authorization refunds and SME incentives.

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ASPHALION: Proven experience in ODD, end-to-end support

45+ orphan drug projects overthe last 5 years, from designation to maintenance of orphan status

45+

ODD and orphan medicine projects

REGIONS
EU, UK & US

TIMEFRAME
Last 5 years

SCOPE
ODD initial designation, maintenance and orphan medicinal products

01 ELIGIBILITY & STRATEGY
Prevalence, seriousness and rationale assessment to define the right route.

02 ODD APPLICATION
Preparation and submission of orphan designation applications.

03 AGENCY INTERACTION
Pre-submission support and responses to agency questions.

04 SIGNIFICANT BENEFIT & SIMILARITY
Development of arguments to support orphan designation and MAA strategy.

05 MAINTENANCE & LIFE-CYCLE
Orphan maintenance, designation updates and transfers.

06 AD-HOC SUPPORT
Flexible help at any stage, from one section to the full dossier.

LET'S TALK! Developing a medicine for a rare disease? We'll help you secure and keep orphan drug status.

Contact us!

info@asphalion.com
www.asphalion.com

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