



RARE DISEASE

Experts. Experience. Execution.

Understanding Orphan Designations and Rare Disease Studies from a Regulatory Perspective

Rare diseases are diseases which affect a small number of people compared to the general population. There are thousands of rare diseases, with 6-7,000 discovered to date and new diseases regularly described in medical literature.

Global Regulatory Expertise

A key component is global regulatory understanding and consultation regarding the unique environment for Rare Disease studies.

Orphan medicines are those intended to treat rare diseases and represent the focus of regulatory agencies in driving state-of-the-art innovation in this area. Regulatory bodies define rare diseases as those that are life-threatening or chronically debilitating conditions.

Medpace's Innovative Approach for Rare Disease and Orphan Drug Projects

Medpace has developed experienced project teams for these complex studies. Integrated processes regarding site relationships with access to patient registries to drive patient enrollment, broad understanding of patient advocacy issues and organizations for support, and noted physicians and pediatricians are critical to a project's success.

What is the Regulatory Environment for Orphan Policies in Key Global Regions?

Country	United States	European Union	Japan
Legal Framework	21 CFR part 316; Orphan Drug Act	Regulation (EC) No 141/2000	Article 77-2 of the Pharmaceutical Affairs Law
Administrative Agency	Food & Drug Administration (FDA) / Office of Orphan Products & Development (OOPD)	European Medicines Agency (EMA) /Committee for Orphan Medicinal Products (COMP)	Ministry of Health, Labour and Welfare (MHLW) / Pharmaceutical Affairs & Food Sanitation Council (PAFSC)
Prevalence	<200,000	<250,000 (5 in 10,000)	<50,000
Estimated Population	326,111,090	506,847,612	126,189,330
Incentives	<ul style="list-style-type: none"> • 7 years market exclusivity • Tax credits • PDUFA fee exemption 	<ul style="list-style-type: none"> • Protocol assistance • Centralised procedure access • 10 years market exclusivity • Fee reductions 	<ul style="list-style-type: none"> • Guidance and consultation from MHLW, NIBIO, PDMA • Preferential tax treatment • Priority review • 10 year market exclusivity
Grants	<ul style="list-style-type: none"> • FDA Office of Orphan Products Development (OOPD) grant program 	<ul style="list-style-type: none"> • Horizon 2020, EU Framework Programme for Research and Innovation (refer to personalizing health and care theme) • E-Rare, a transnational project for research programmes on rare diseases • International Rare Disease Consortium (IRDIRC) 	<ul style="list-style-type: none"> • Subsidies through National Institute of Biomedical Innovation (NIBIO) for development
Accelerated Marketing Procedure	Yes	Yes	Yes

Orphan Drug Designation Focus Areas

- Clear concise description of the disease or condition in question based on literature evidence, including causes and symptoms as appropriate
- Rationale for use of medicinal products in the proposed orphan designation including a discussion of its mechanism of action, pre-clinical, and clinical development
- Justification for the life-threatening or debilitating nature of the proposed condition
- Demonstration of the prevalence in accordance with defined criteria using authoritative references and relevant epidemiological data
- Discussion of existing medicinal products available for diagnosis, prevention, or treatment of the proposed orphan designation, including justification as to why existing medicinal products are not considered satisfactory, as appropriate
- Justification of the significant benefit of the proposed medicinal product
- Review of current development status including plans in place for the intended orphan indication
- Overview of its marketing status, as applicable

Medpace Services

Medpace Global Regulatory Affairs have a wealth of experience in supporting our customers through the global orphan designation pathway. Our experts are available to assist in the following areas:

- Education on regional/country nuances
- Development planning for orphan indications
- Full submission package support addressing key focus areas per region
- Review and gap analyses of customer prepared packages, offering guidance as required to ensure successful outcomes
- Full regulatory agency interaction through to conclusion of orphan procedure
- Guidance and support for pre-submission activities with regulatory agencies
- Resolution of regulatory agency comments
- Holding orphan designations on behalf of customers without a legal presence

Medpace Global Regulatory Affairs

Medpace Global Regulatory Affairs, led by Steven B. Johnson, Vice President, is a world-class, stage-setting, and professional regulatory affairs and medical writing organization that facilitates rapid development of safe and effective therapeutics for our clients. Our team is comprised of more than 50 experts providing coverage across all areas of clinical development through marketing authorization.

About Medpace

Medpace is a scientifically-driven, global full-service clinical contract research organization (CRO) providing Phase I-IV clinical development services for drug, biologic, and device programs. Medpace's physician-led, high-science, and disciplined operating approach leverages regulatory and therapeutic expertise to accelerate the global development of safe and effective medical therapeutics across all major areas including oncology, cardiovascular, metabolic/diabetes, infectious disease, and neuroscience.

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