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Global regulations governing orphan drug designation

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Rare disease definitions and orphan drug designation (ODD) requirements vary across jurisdictions, shaping development strategy and evidence needs. This article examines orphan drug designation frameworks in the US, EU, UK, Japan, and China, highlighting differences in eligibility criteria, timelines, and incentives. It discusses how these differences affect evidence generation, sequencing of submissions, and long-term global planning for orphan medicines.

Keywords – orphan drug designation, rare disease definition, regulatory policy

Introduction

Rare disease definitions and ODD frameworks vary across regulatory jurisdictions, reflecting differences in regulatory philosophy, evidentiary standards, and health system context. Although a recent initiative by a multistakeholder panel of experts has developed a consensus on an operational definition of a rare disease,[1] no universally harmonized global definition exists, and regional frameworks remain the primary basis for regulatory classification and policy implementation.[2]

ODD is the principal instrument used to support therapeutic development for rare diseases. While ODD frameworks share a common objective across regions, they differ substantially in eligibility criteria, evidentiary expectations at the designation stage, and the structure and timing of regulatory incentives.[3-7] These differences extend beyond numerical prevalence thresholds and include jurisdiction-specific regulatory constructs, such as acceptance of orphan subsets, requirements to demonstrate comparative benefit, feasibility assessments, and reliance on formal disease catalogs. Such divergences have important implications for orphan drug development, influencing designation feasibility, early evidence generation, and regulatory engagement strategies across regions.

This article examines rare disease definitions and orphan drug designation frameworks in the US, EU, UK, Japan, and China, and how differing regulatory constructs shape global orphan drug development strategies. The article also explores how jurisdiction-specific ODD frameworks influence development feasibility and strategic decision making. By situating regulatory definitions within their broader health-system context, this article seeks to provide a practical understanding of how regulatory design affects downstream patient access.

Definitions and regulatory classification

Regulatory definitions of rare diseases underpin orphan drug designation frameworks and determine eligibility for regulatory incentives. Despite having shared policy objectives, regulatory authorities apply distinct conceptual criteria to define rarity, including prevalence thresholds, diagnostic requirements, and regulatory constructs such

as orphan subsets, feasibility expectations, and catalog-based eligibility (**Table 1**).

As a result of these differing definitions and criteria, the same condition may qualify as rare in one jurisdiction but not in another, independent of epidemiology alone. These differences establish the regulatory context within which orphan designation decisions are made.

Regulatory approaches to defining rare diseases

Across jurisdictions, regulatory authorities employ differing conceptual approaches to defining rare diseases. These include acceptance of orphan subsets, requirements for comparative benefit, feasibility expectations, and reliance on formal disease catalogs (Table 1). While prevalence thresholds determine formal eligibility, these embedded regulatory concepts play a more decisive role in designation feasibility.

Implications of divergent regulatory definitions

In the US, the Food and Drug Administration (FDA) emphasizes developmental flexibility by permitting orphan subsets and accepting limited earlystage evidence,

Table 1. Rare disease definitions and requirements across major regulatory jurisdictions[3-11]

FDA	EMA	MHRA	MHLW/PMDA	NHC/NMPA
Definition of rare disease				
Disease affecting <200,000 people or costs cannot be reasonably recovered	Life-threatening or chronically debilitating condition affecting <5/10,000 people or insufficient market return	Life-threatening or chronically debilitating condition affecting <5/10,000 people	Condition affecting <50,000 people nationally	Disease defined by inclusion in the National Rare Disease Catalogue
Prevalence threshold[a]				
<200,000	<5/10,000	<5/10,000	<50,000	<1/10,000 prevalence or <1/10,000 newborn incidence
Special criteria				
Accepts scientifically justified orphan subsets; flexible early-stage evidence	Requires significant benefit unless no satisfactory therapy exists	Requires significant benefit unless no satisfactory therapy exists; aligned with EMA	Requires strong development feasibility and supporting data	Requires clear diagnostic criteria; eligibility tied to catalog inclusion
Use of rare-disease list				
No centralized list	No unified list; previous orphan designations can be found in the community register of orphan medicinal products	No unified list; aligns with EMA principles	Uses the Nanbyo[b] list	Uses the National Rare Disease Catalogue

FDA, Food and Drug Administration [US]; **EMA**, European Medicines Agency; **MHRA**, Medicines and Healthcare products Regulatory Agency [UK]; **MHLW**, Ministry of Health, Labour and Welfare [Japan]; **NHC**, National Health Commission [China]; **NMPA**, National Medical Products Administration [China].

[a] Prevalence refers to the number of individuals affected by the disease within a given population. For diagnostic claims, prevalence is measured as the number of people diagnosed per year. For prevention claims, prevalence includes all individuals at risk of developing the disease. [b]The Nanbyo list currently includes 338 intractable designated conditions. Diseases already covered by separate national frameworks, such as cancer, mental illness, and infectious diseases, are excluded from this list.

enabling earlier designation and regulatory engagement.[3,8] In contrast, the EU and the UK introduce comparative evidence requirements at the designation stage through the significant-benefit criterion, increasing evidentiary expectations earlier in development.[4,5] In this context, the *significant-benefit criterion* requires that, where a satisfactory method of diagnosis, prevention, or treatment has already been authorized, the sponsor demonstrates that the designated product is expected to confer a clinically relevant advantage or make a major contribution to patient care.[4,5]

Japan's framework requires a theoretical rationale for the use of the product for the target population, and an appropriate development plan.[6,7] This should be supported by existing nonclinical and Phase 1/2 data, unless the product is approved overseas or sufficient clinical study data are available. In China, the latest definition of rare disease allows for the inclusion or removal of conditions in China's National Rare Disease Catalog. However, this list can constrain early regulatory engagement for newly characterized or ultra-rare conditions that have not yet been formally recognized.[9-11]

These regional structural differences influence not only whether a condition qualifies for orphan designation, but also the timing of applications, the nature of evidence generated, and the sequencing of regulatory submissions across regions.

Incentives, market exclusivity, and pathways

Beyond the differing definitions and requirements of ODDs shown in Table 1, ODD frameworks across jurisdictions also vary widely regarding the incentives offered, market exclusivity periods, regulatory support offered, pathways, and official registers (**Table 2,** p. 7). It should be noted that some EU incentives and exclusivity provisions may change under the new EU General Pharmaceutical Legislation.

Proposed changes in the EU

The proposed EU General Pharmaceutical Legislation, which includes a new directive on the EU code and a new regulation laying down EU procedures, would introduce several changes affecting orphan designation, regulatory decision making, and market exclusivity.[12,13] Under the proposed legislation, the orphan designation criterion based on return on investment would be removed; however, the practical effect is expected to be limited, as EU orphan designation has relied mainly on the prevalence threshold and the no satisfactory method/significant-benefit criterion.

The draft legislation would shift final decision making on orphan designation from the European Commission to the European Medicines Agency (EMA). Under the legislation, decisions on granting, refusing, or transferring orphan designation would no longer follow the current model, in which the Committee for Orphan Medicinal Products (COMP) adopts an opinion, and the European Commission issues the final decision. This change is linked to the broader reform of EMA committee structures, including the replacement of the current committee's configuration by new working groups.

The proposed reform would also change how orphan market exclusivity operates in the EU:

- A company would no longer receive separate orphan exclusivity periods for multiple marketing authorizations covering the same active substance;
- An additional 12 months of exclusivity could still be earned if the product later obtains approval for a new orphan indication in a different orphan condition, although this extension would be limited and granted only under specified conditions; and
- Applications for similar products, including generics and biosimilars, could be submitted and reviewed before the orphan exclusivity period has fully expired, when less than two years of exclusivity remain, which would reduce the practical period of protection.

Another proposed change concerns the validity of orphan designation, which currently has unlimited validity, but under the new rules would expire after seven years. For existing orphan products, this validity period would begin when the regulation comes into force. Based on the current implementation timetable, the adopted acts are expected to enter into force in December 2026, with a transition period running to 2028; accordingly, for existing orphan products, this change would be expected to start applying from 2028. The validity may be extended upon a justified request if the sponsor can provide evidence that the relevant studies supporting the use of the designated orphan medicinal product in the intended conditions are ongoing and promising for filing a future marketing authorization application.

This proposed change is intended to encourage faster

Table 2. Incentives, market exclusivity, support, and pathways for ODDs across jurisdictions

FDA	EMA[a]	MHRA	MHLW/ PMDA	NHC/NMPA
Available grants/programs				
OOPD grants for clinical trials	ERDERA IRDiRC grants	No direct, research grants	Government grants/subsidies via NIBIOHN	Incentives for catalog-listed diseases
Tax incentives				
Up to 50% tax credit for clinical trial costs	Member states may offer tax incentives (none at EU level)	No direct tax incentives	12% tax credit on qualified clinical study expenditures	Reduced VAT (3% vs. 16%) and import taxes for qualifying drugs
Financial and fee incentives				
Waiver of application fees (e.g., PDUFA)	Fee waivers/ reductions for protocol assistance and MAA	– Partial/full MAA fee waiver/ refund (100% for SMEs) – Full fee waiver for UK-based SMEs for scientific advice	Lower fees for consultations and orphan drug review	– Additional R&D cost deductions – SME fee waivers
Market exclusivity				
7 years for first approved orphan indication	10 years (extendable to 12 years with PIP)[b]	10 years (extendable by 2 years with PIP)	10-year re-examination period	7 years plus 6 years of data protection for innovative drugs (draft)
Scientific and regulatory support				
– Scientific advice – Protocol assistance – Rare pediatric disease priority review voucher	Protocol assistance	Scientific advice for significant benefit demonstration	– Mandatory presubmission consultation – Robust foreign data – Accepted priority consultation	Technical guidance
Accelerated pathways				
– Fast track – Breakthrough therapy – Priority review – Accelerated approval	– Reduced MAA review – Centralized authorization – Conditional approval	– Parallel ODD and MA evaluation – Accelerated/ conditional approval for high-promise medicines	– Priority review – Conditional early approval for innovative drugs	– Accelerated review for urgent needs – Conditional approval when justified

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Table 2. (*cont.*) Incentives, market exclusivity, support, and pathways for ODDs across jurisdictions

FDA	EMA[a]	MHRA	MHLW/ PMDA	NHC/NMPA
Orphan register				
Public Orphan Drug Product Designation Database	EU Community Register of Orphan Medicinal Products	GB Orphan Register	List of designated orphan drugs	No single official orphan list

EMA, European Medicines Agency; **ERDERA**, European Rare Diseases Research Alliance; **FDA**, Food and Drug Administration [US]; **IRDiRC**, International Rare Diseases Research Consortium; **MAA**, marketing authorization application; **MHLW**, Ministry of Health, Labour and Welfare [Japan]; **MHRA**, Medicines and Healthcare products Regulatory Agency [UK]; **NHC**, National Health Commission [China]; **NIBIOHN**, National Institute of Biomedical Innovation, Health and Nutrition [Japan]; **NMPA**, National Medical Products Administration [China]; **ODD**, orphan drug designation; **OOPD**, Office of Orphan Products Development; **PDUFA**, Prescription Drug User Fee Act; **PIP**, Paediatric Investigation Plan; **PMDA**, Pharmaceuticals and Medical Devices Agency [Japan]; **SME**, small and medium-sized enterprise; **VAT**, value-added tax.

[a]According to proposed pharmaceutical legislation (status as of May 2026), where noted. [b]See section on proposed changes in the EU.

development and authorization of designated orphan products; however, it could also lead companies to apply for orphan designation later in the development process. In practice, a later application may allow sponsors to preserve more of the designation validity period closer to the time of marketing authorization, but it may also reduce the opportunity to benefit from orphan-specific incentives, such as protocol assistance, during earlier stages of development. To increase predictability for developers, the possibility of reviewing the eligibility criteria for market exclusivity after six years of the marketing authorization will be abolished once the proposed regulation is in force.[12]

Overall, these changes reduce the relative attractiveness of orphan designation in the EU compared with previous frameworks and other major jurisdictions because they narrow the practical benefit of exclusivity, limit lifecycle advantages across multiple orphan indications, and may weaken incentives for early designation.

China and Japan

China's system is functionally similar to those of the US and the EU in terms of outcomes but differs in structure (**Table 3,** p. 9). China has no single Orphan Drug Act; instead, it operates a rare-disease drug framework built upon the National Rare Disease Catalog, accelerated regulatory pathways, and market exclusivity. In effect, China recognizes orphan drugs, but eligibility is not tied to a named ODD label.

China's Drug Administration Regulations were revised in 2026.[1] One of the new elements relates to eligible treatments of rare diseases, allowing for the possibility of a market exclusivity period of up to seven years. This possibility is contingent upon the marketing authorization holder (MAH) committing to a guaranteed drug supply. If the MAH fails to fulfill its supply obligations, the exclusivity will be revoked.[14]

In contrast, Japan applies a formal ODD, which is granted under the MHLW/PMDA framework, generally for serious conditions affecting fewer than 50,000 Japanese patients and supported by evidence of development feasibility, clinical need, and population relevance.[6,7] Japan also offers a structured package of incentives linked to designation, including subsidies, tax credits, priority consultation and review, and a re-examination period that functions as postauthorization protection.[6,7]

Table 3 compares China and Japan's ODD structures with those of the US and the EU. A UK comparison was intentionally omitted due to its similarity with the EU.

Transparency

To determine where to apply for orphan designation and the sequence of designations, manufacturers should consider transparency regarding the submitted data as a key factor.

A key difference between the EU and the other regions is that an orphan designation request application with minimal redactions can be made available to interested parties. The EMA's policy on access to documents describes the rules the agency applies when granting access to documents it holds

Table 3. China's and Japan's ODD system architectures compared with US and EU

NHC/NMPA	MHLW/PMDA	FDA	EMA
Legal foundation			
– No single orphan drug act – Orphan status embedded in drug administration law and implementing regulations	– Designation system under the Pharmaceuticals and Medical Devices framework – Designation granted by MHLW with PMDA review support	Orphan Drug Act (1983), a single, dedicated statute	EU Orphan Regulation (EC No 141/2000)
Rare disease definition			
Included in the Rare Disease Catalogue issued by the National Health Commission	–Generally <50,000 people in Japan – Linked to serious disease and high unmet medical need	<200,000 people in the US	≤5 in 10,000 people in the EU
Formal orphan designation label			
No unified label	Yes (orphan drug designation)	Yes (orphan drug designation)	Yes (orphan medicinal product)
How incentives are applied			
Incentives applied at drug level through pathways	Incentives applied at product designation level with linked financial, review, consultation, and re-examination incentives	Incentives applied at product designation level	Incentives applied at product designation level

EMA, European Medicines Agency; **FDA,** Food and Drug Administration [US]; MHLW, Ministry of Health, Labour and Welfare [Japan]; **NHC,** National Health Commission [China]; **NMPA,** National Medical Products Administration [China]; **ODD,** orphan drug designation; **PMDA,** Pharmaceuticals and Medical Devices Agency [Japan].

on human and veterinary medicines and documents not related to medicines.[15] This can include documents produced by the EMA (e.g., opinion on orphan designation, COMP summary report, written comments from COMP members, and lists of questions) as well as the scientific part of the application prepared by the applicant.

Although the EMA allows limited redaction of commercially confidential information, this is a concern for some companies.[16] Public disclosure may expose elements of a sponsor's prevalence calculations, including how data sources are combined and whether commercial registries are used, as well as the planned approach for generating comparative evidence to support significant benefit at the time of marketing authorization. While the EMA permits redaction, sponsors may need to justify each proposed redaction under the applicable legal exceptions. This can be resource-intensive, and in some cases, proposed redactions are not accepted.

In the US, it is possible to request information on orphan designations from the FDA under the Freedom of Information Act, and indeed, in some cases, orphan drug designation requests have also been made.[17] However, contrary to the EMA, large portions of these orphan designation requests are often heavily redacted by the FDA due to confidentiality protections. In Japan, China, and the UK, the data supporting orphan designation applications are not made public or are disclosed only after the marketing authorization is granted.

Cell and gene therapies

Cell, gene, and combined ex vivo gene-modified cell therapies (CGT) are eligible for orphan incentives across the major jurisdictions, but they are not framed identically in regulatory law.[4,6,7,18-20] In Japan, cell and gene therapies may fall within the dedicated category of regenerative medical products under the Pharmaceuticals and Medical Devices Act, to which orphan designation can apply.[6,7,19] In contrast, the US and China generally regulate these products within drug/biologic pathways, while the EU classifies many of them as advanced therapy medicinal products (**Table 4**).[4,18,20]

As shown in Table 4, the way cell and gene therapies are classified and incorporated into orphan-drug frameworks is broadly similar across regions. However, Japan has a

Table 4. Eligibility and positioning of cell and gene therapies across jurisdictions

NHC/NMPA	MHLW/PMDA	FDA	EMA
Regulatory classification			
Drugs/biological products	Regenerative medical products for many cell and gene therapies; some products may also be regulated as drugs/biologics depending on modality	Drugs/biologics	ATMPs (gene, cell, tissue)
Eligible for orphan incentives			
Yes, if the target disease is included in the rare disease framework	Yes, including orphan-designated regenerative medical products	Yes	Yes
CGT-specific orphan criteria at designation stage			
Rare Disease Catalogue inclusion (or equivalent NHC recognition) and diagnostic criteria	– Less than 50,000 patients, high medical need, and an appropriate development plan – Criteria explicitly extend to regenerative medical products	– Scientifically justified orphan subsets may be accepted – Flexible early-stage evidence	Significant benefit is required unless no satisfactory therapy exists
CGT-specific guidance			
Yes (CDE technical guidelines)	Yes (PMDA/MHLW regenerative medical product guidance, including conditional and time-limited approval guidance)	Yes (gene therapy orphan guidance)	Yes (ATMP and orphan guidance)
Market exclusivity/postapproval protection			
Up to 7 years	10-year re-examination period for approved orphan drugs and orphan regenerative medical products	7 years	10 years (12 years for pediatrics)

ATMP, advanced therapy medicinal products; **CGT**, cell and gene therapy; **CDE**, Centre for Drug Evaluation; **EMA**, European Medicines Agency; **FDA**, Food and Drug Administration [US]; **MHLW**, Ministry of Health, Labour and Welfare [Japan]; **NHC**, National Health Commission [China]; **NMPA**, National Medical Products Administration [China]; **PMDA**, Pharmaceuticals and Medical Devices Agency [Japan].

distinct regenerative medicine framework, and China remains more dependent on rare-disease catalog recognition for orphan-linked positioning.[6,7,9-11,19,20] The table does not include the UK, which has a very similar system to the EU.[4,5,21]

A practical global roadmap

A practical global orphan-drug roadmap should align the timing of designation requests, the design of evidence packages, and regulatory engagement plans with the distinct requirements of each jurisdiction.

- **Sequencing**: Begin in flexible jurisdictions (i.e., US, Japan) to secure early designation, then prepare stronger comparative evidence for the EU/UK. In addition, EU transparency and the possibility that orphan designation would remain valid for seven years (unless extended) support applying later in the EU.
- **Evidence modularity**: Build modular packages (epidemiology, diagnosis validity, natural history, comparative benefit, and bridging data) to meet different regional requirements.
- **Policy resilience**: Plan for evolving exclusivity rules and regulatory reforms (notably in the EU and China) by incorporating scenario planning early.

In practice, this means using early regulatory interactions in the US and Japan to establish feasibility and development momentum, while preparing the stronger comparative and diagnostic evidence needed for the EU, the UK, and China. A roadmap built on sequencing, evidence modularity, and policy resilience is more likely to preserve optionality across regions and reduce the risk that a product qualifies for orphan incentives in one jurisdiction but not in another.

Conclusion

No globally harmonized definition of rare disease exists, and orphan drug designation outcomes are primarily shaped by jurisdiction-specific regulatory constructs rather than prevalence thresholds alone. The most influential divergence occurs at the designation stage, where Europe's significant-benefit requirement and China's catalog-anchored eligibility introduce early comparative and diagnostic constraints not observed in the US or Japan.

Over the past five years, orphan drug development has remained a major driver of regulatory innovation in the US, while China has demonstrated rapid alignment through regulatory reform, catalog expansion, and growing acceptance of multiregional clinical data. Japan continues to offer substantial lifecycle value for orphan products when feasibility and population-specific planning are addressed early.

As regulatory harmonization remains unlikely in the near term, effective global orphan strategies combine region-specific planning, including flexible early-stage development, comparative evidence generation, and alignment with local regulatory requirements. Differences in orphan drug designation frameworks also support a region-specific sequencing strategy, particularly regarding the timing of designation applications and evidence development.

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