Orphan Drug Development Guidebook

Building Block J301

This document defines the content of the Building Block created for each identified tool, incentives, initiative or practice introduced by public bodies or used by developers to expedite drug development in Rare Diseases (RDs).

ITEM	DESCRIPTION
Building Block (BB) Title	Japanese Orphan Drugs/Medical Devices/Regenerative Medical Products Designation
References	● MHLW's website: Overview of Orphan Drug/Medical Device Designation System – https://www.mhlw.go.jp/english/policy/health-medical/pharmaceuticals/orphan_drug.html
Description	In Japan, drugs, medical devices, and regenerative medical products can be designated as orphan drugs, medical devices, or regenerative medical products based on the Article 77-2 of the Act on Securing Quality, Efficacy and Safety of Pharmaceuticals, Medical Devices if they are intended for use in basically less than 50,000 patients in Japan and for which there is a high medical need. They are designated by the Minister of Health, Labour and Welfare based on the opinion of the Pharmaceutical Affairs and Food Sanitation Council (PAFSC). Designation of orphan drugs/medical devices/regenerative medical products does not automatically lead to marketing approval. For designated orphan drugs/medical devices/regenerative medical products, measures to support the research and development activities are taken. The cost of procedure is free of charge.
Category	Regulatory Building Block
Geographical scope	Japan

ITEM	DESCRIPTION
Availability	Applicants developing drugs/ medical devices/ regenerative medicinal products for rare diseases.
Scope of use	All matters related to orphan drugs/medical devices/regenerative medical products designation and regulations for pharmaceutical companies
Stakeholders	• Drugs/Medical Devices/Regenerative Medical Products developers • Ministry of Health, Labour and Welfare (MHLW) • Pharmaceuticals and Medical Devices Agency (PMDA) (preliminary review on the eligibility and reports back to the MHLW) • Pharmaceutical Affairs and Food Sanitation Council (PAFSC) (approval delivered by the MHLW is based on discussions with the PAFSC) • National Institute of Biomedical Innovation, Health and Nutrition (NIBIOHN) (providing funds for R&D to the applicant)
Enablers/ Requirements	MHLW may designate orphan drugs/medical devices/regenerative medical products if the following criteria are fulfilled: • The number of patients must be less than 50,000 in Japan, or the disease has to be designated as Nan-byo. • The drug/medical device/regenerative medical product is considered high priority in healthcare needs • The drug/medical device/regenerative medical product has a possibility of development
Output	Significant help for orphan drug development and marketing exclusivity after registration within Japan • Priority consultation on clinical trials and priority review • Discount on consultation and review fees (only for designated orphan drugs) • Extension of re-examination period (marketing exclusivity) • Grant for R&D

ITEM	DESCRIPTION
	• Tax incentives
Best time to apply and time window	Anytime during the drug development before MAA (Marketing Authorization Application) submission. Possibility of development needs to be explained. 1 Phase of Designation Consultation in MHLW (a few months) 1.1 Submission of the application for designation consultation 1.2 Arrangement for the consultation date 1.3 Designation consultation 2 Phase of Evaluation for Designation in MHLW/PMDA (several months) 2.1 Submission of the application for designation in MHLW 2.2 Review of eligibility for an orphan drugs/medical device/regenerative medical products designation in MHLW 2.2.1 Preliminary evaluation of eligibility in PMDA 2.3 Committees of PAFSC in MHLW 2.4 Designation in MHLW
Expert tips	Application documents need to be created in Japanese. PROs: • Priority consultation on clinical trials and priority review • Discount on consultation and review fees (only for designated orphan drugs) • Extension of re-examination period (marketing exclusivity) • Grant for R&D • Tax incentives CONs: N/A