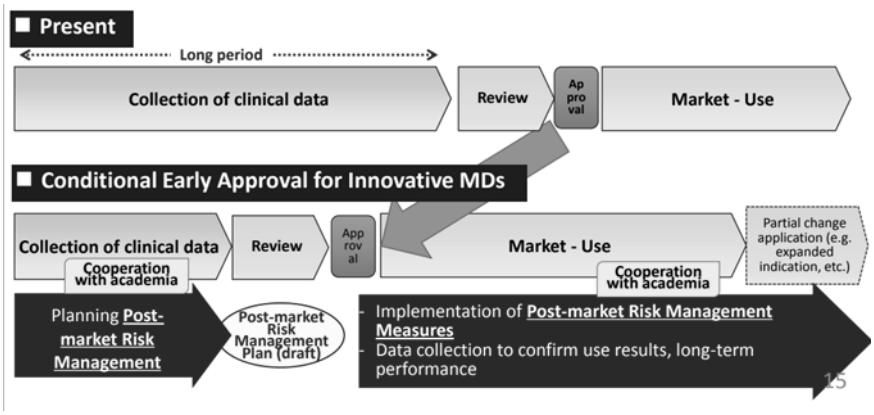
Orphan Drug Development Guidebook

Building Block J307

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	Conditional Early Approval for Medical Devices
References	• New Regulatory Framework for Medical Devices in Japan: Current Regulatory Considerations Regarding Clinical Studies – https://www.pmda.go.jp/files/000227090.pdf – https://www.ncbi.nlm.nih.gov/pubmed/25842974 • PMDA Asia Training Center Learning Videos: Review 9. Expedited Regulatory Pathways in Japan) – https://www.pmda.go.jp/english/int-activities/training-center/0005.html • PMDA website: User Fees (Japanese) – https://www.pmda.go.jp/review-services/drug-reviews/user-fees/0001.html
Description	Conditional Early Approval System is a system to put highly useful and effective medical devices for treating serious diseases into practical use as early as possible. 

ITEM	DESCRIPTION
Category	Regulatory Building Block
Geographical scope	Japan
Availability	Medical devices developers for rare and non-rare diseases that have marketing licenses within Japan
Scope of use	To provide patients with early access of medical devices for unmet medical needs
Stakeholders	• Pharmaceuticals and Medical Devices Agency (PMDA) • Ministry of Health, Labour and Welfare (MHLW) • Relevant academic medical societies • Medical devices developers
Enablers/ Requirements	Clinical evidence not confined to rigorous prospective randomized controlled trials, but including other adequate clinical data reasonably likely to predict clinical benefit and safety (case studies, registries, and/or clinical researches) based on a limited patient population in certain clinical settings. Requirement (i) There are no appropriate alternative treatment or there is a reasonable likelihood of greater efficacy and safety compared with existing products (ii) The target patient population is affected by life-threatening disease or irreversible disease with serous disability in daily life (iii) A certain extent of supporting clinical evidence is available

ITEM	DESCRIPTION
	(iv) There is a post marketing commitment to an appropriate risk-management plan in collaboration with relevant academic medical societies and rigorous real-world evidence collection and evaluation (v) There is justification of difficulty in conducting a new prospective clinical trial
Output	• A shortened time for approval of the medical device and an early access to patients
Best time to apply and time window	After exploratory clinical trials
Expert tips	Recommend to use PMDA consultation or Regulatory Science General Consultation and Regulatory Science Strategy Consultation (R&D) PROs: The Conditional Early Approval for Medical Devices provides patients with early access of medical devices in need. CONs: N/A