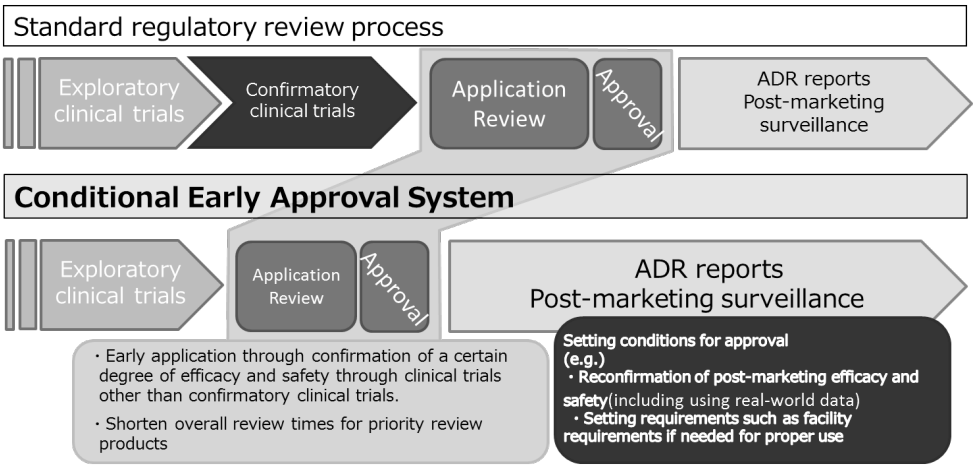
Orphan Drug Development Guidebook

Building Block J306

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 System for Drugs
References	• Regulatory Update from MHLW/PMDA (5th Joint Conference of Taiwan and Japan on Medical Products Regulation) – https://www.pmda.go.jp/files/000221880.pdf • PMDA website: User Fees – 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 drugs for treating serious diseases into practical use as early as possible. [Candidate product] -Drugs that treat serious diseases for which there are limited treatment options and, -Drugs that it is difficult to conduct clinical trials or it takes long period because the number of patients is small [Requirement] MHLW/PMDA needs to - Confirm a certain degree of efficacy and safety through clinical trials other than confirmatory clinical trials at the time of submission - Clarify management of conditions for approval such as imposing to conduct research which is necessary for reconfirmation of post-marketing efficacy and safety

ITEM	DESCRIPTION
	
Category	Regulatory Building Block
Geographical scope	Japan
Availability	Drug developers for both rare and non-rare diseases that have marketing licenses within Japan
Scope of use	To provide patients with early access of drugs for unmet medical needs.
Stakeholders	• Ministry for Health, Labor and Welfare • Pharmaceuticals and Medical Devices Agency • Drug developers
Enablers/ Requirements	Drugs eligible for the system should meet the all requirements from 1 to 4 listed below. 1. Seriousness of indications (i) Diseases which have significant impact on lives (life-threatening diseases), (ii) Progress of disease is irreversible and the disease has a significant impact on daily lives, or (iii) Others

ITEM	DESCRIPTION
	2. Medical usefulness (i) No existing remedies, preventive therapies or diagnostics, or (ii) medical usefulness is better than that of existing remedies, preventive therapies or diagnostics in terms of efficacy, safety, and patient's physical and mental burden 3. Being difficult to conduct confirmatory clinical trials or considered to take considerable time to complete trials because of a limited number of patients 4. Considered to have of a certain degree of efficacy and safety through clinical trials other than confirmatory clinical trials
Output	The Conditional Early Approval System for Drugs allow a shorter overall review time for drugs, for the early access of medicines to patients in need.
Best time to apply and time window	When a certain degree of efficacy and safety are confirmed through clinical trials other than confirmatory 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 System for Drugs allow a shorter overall review time for drugs, for the early access of medicines to patients in need CONs: N/A