
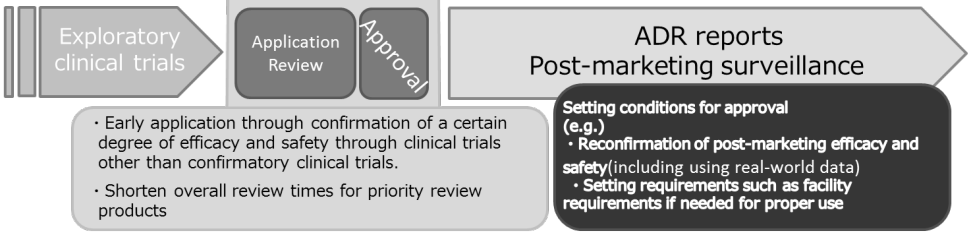


Orphan Drug Development Guidebook

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	<ul style="list-style-type: none"> • PMDA Asia Training Center Learning Videos: Review 9. Expedited Regulatory Pathways in Japan <ul style="list-style-type: none"> - https://www.pmda.go.jp/english/int-activities/training-center/0005.html • Notification: Implementation of a Conditional Early Approval System for Pharmaceutical Products <ul style="list-style-type: none"> - https://www.pmda.go.jp/files/000227089.pdf • PMDA website: User Fees (Japanese) <ul style="list-style-type: none"> - https://www.pmda.go.jp/review-services/drug-reviews/user-fees/0001.html
Description	<p>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. This system was introduced in 2017 and established as legislation in 2019.</p> <p>[Candidate product]</p> <ul style="list-style-type: none"> -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 <p>[Requirement]</p> <p>MHLW/PMDA needs to</p> <ul style="list-style-type: none"> - Confirm a certain degree of efficacy and safety through clinical trials other than confirmatory clinical trials at the time of submission

ITEM	DESCRIPTION
	<p>- Clarify management of conditions for approval such as imposing to conduct research which is necessary for reconfirmation of post-marketing efficacy and safety</p> <div style="border: 1px solid black; padding: 10px; margin: 10px 0;"> <p>Standard regulatory review process</p>  </div> <div style="border: 1px solid black; padding: 10px; margin: 10px 0;"> <p>Conditional Early Approval System</p>  <div style="border: 1px solid black; padding: 5px; margin: 5px 0;"> <p>• Early application through confirmation of a certain degree of efficacy and safety through clinical trials other than confirmatory clinical trials.</p> <p>• Shorten overall review times for priority review products</p> </div> <div style="border: 1px solid black; padding: 5px; margin: 5px 0;"> <p>Setting conditions for approval (e.g.)</p> <ul style="list-style-type: none"> • Reconfirmation of post-marketing efficacy and safety (including using real-world data) • Setting requirements such as facility requirements if needed for proper use </div> </div> <p>Fees of some review categories for a drug might be applied.</p>
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 to drugs for unmet medical needs
Stakeholders	<ul style="list-style-type: none"> • Ministry of Health, Labour and Welfare (MHLW) • Pharmaceuticals and Medical Devices Agency (PMDA) • Drug developers
Enablers/ Requirements	Drugs eligible for the system should meet the all requirements from 1 to 4 listed below.

ITEM	DESCRIPTION
	<p>1. Seriousness of indications</p> <p>(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</p> <p>2. Medical usefulness</p> <p>(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</p> <p>3. Being difficult to conduct confirmatory clinical trials or considered to take considerable time to complete trials because of a limited number of patients</p> <p>4. Considered to have of a certain degree of efficacy and safety through clinical trials other than confirmatory clinical trials</p>
Output	The Conditional Early Approval System for Drugs allow a shorter overall review period 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	<p>Recommend to use PMDA consultation or Regulatory Science General Consultation and Regulatory Science Strategy Consultation (R&D)</p> <p>PROs: The Conditional Early Approval System for Drugs allows a shorter overall review time for drugs, for the early access of medicines to patients in need</p> <p>CONs: N/A</p>