Orphan Drug Development Guidebook

Building Block J311

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	Japan Compassionate Use Programs
References	• PMDA Website and related notification: Jindo-Teki Kenchi Kara Jisshi Sareru Chiken • https://www.pmda.go.jp/review-services/trials/0016.html **(Japanese)** • https://www.pmda.go.jp/files/000227843.pdf (English)
Description	A system for patients with life-threatening conditions without effective a treatment to gain access to unapproved investigational medical products. For investigational medical products that are undergoing clinical trials that meet certain criteria, such as those that targets are serious diseases but do not have alternative treatment methods, incorporate them within acceptable limits for patients who do not satisfy inclusion/exclusion criteria and ask the pharmaceutical company to conduct clinical trials that relax standards in parallel. Summary of conducting a clinical trial based on Japan compassionate use programs 1. A Developer receives a request of a clinical trial based on Japan compassionate use programs from physicians 2. The developer considers and makes a decision of a clinical trial based on Japan compassionate use programs by the developer 3. The developer submits a clinical trial notification from medical product developers to MHLW/PMDA

	4. The developer starts a clinical trial based on Japan compassionate use programs Related to costs, basically, medical product developers bear the cost of a clinical trial based on Japan compassionate use program. However, it is possible to ask patients enrolled in a trial for paying some expense related to investigational products.
Category	Early Access Building Block
Geographical scope	Japan
Availability	Applicants developing medicines for rare and non-rare diseases.
Scope of use	Providing patients with access to unapproved investigational medical products with balancing its risk and benefit.
Stakeholders	• Medical product developer of drugs, medical devices and regenerative medicinal products • Ministry of Health, Labour and Welfare (MHLW)/Pharmaceuticals and Medical Devices Agency (PMDA) • Patients
Enablers / Requirements	Clinical trial notification needs to be submitted to MHLW/PMDA same as a standard clinical trial
Output	Providing patients with access to unapproved investigational medical products
Best time to apply and time window	During or after clinical trial(s) in the final stage of development (After completion of subject enrollment)
Expert tips	Refer to Information in Japanese