Orphan Drug Development Guidebook

Building Block J303

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	PMDA consultations
References	• PMDA Profile of Services – https://www.pmda.go.jp/english/about-pmda/outline/0005.html • PMDA Website: Consultations – https://www.pmda.go.jp/english/review-services/consultations/0002.html • PMDA Website: User Fees – https://www.pmda.go.jp/review-services/drug-reviews/user-fees/0001.html (Information of consultation fees, Japanese)
Description	PMDA offers consultations to give guidance and advice on clinical trials of drugs, medical devices, and regenerative medical products as well as on data for regulatory submissions. For designated orphan drugs, PMDA offers Priority consultation.

ITEM	DESCRIPTION
Category	Regulatory Building Block
Geographical scope	Japan
Availability	Developers within Japan regardless of the applicant's nature (i.e., public/no-profit/academia, SME, etc). Priority consultation is offered to designated orphan drugs.
Scope of use	In clinical trial consultations for drugs, medical devices, and regenerative medical products, PMDA checks whether a proposed clinical trial complies with the requirements for regulatory submission, taking into consideration the ethical and scientific aspects and reliability of the clinical trial as well as the safety of trial subjects, and also gives advice that leads to an improvement in the quality of the clinical trial.
Stakeholders	• Drug/Medical device/Regenerative Medical Product developers • Pharmaceuticals and Medical Devices Agency (PMDA)
Enablers/ Requirements	N/A
Output	Scientific advice with meeting minutes from PMDA
Best time to apply and time window	Anytime during medical product development. Various types of consultations are available. Summary of applicant's procedure (It takes approximately three months from application to receiving minutes) **1.** Application of consulation **2.** Submission of consulation materials **3.** Receiving inquiry from PMDA and submission of response 4. Receiving preliminary opinion from PMDA and submission of response **5.** Face-to-Face meeting with PMDA

ITEM	DESCRIPTION
	6. Receiving the minutes of consulation from PMDA
Expert tips	PROs: Provision from PMDA for appropriate quality, preclinical and clinical development (i.e., tests and studies) required in the development of a drug/medical device/regenerative medical product is obtained. If the advices are followed or justified, this should minimize objections at the time of MAA evaluation which could delay or compromise the registration. CONs: N/A