Orphan Drug Development Guidebook

Building Block J304

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	Regulatory Science General Consultation and Regulatory Science Strategy Consultation (R&D)
References	• PMDA Profile of Services – https://www.pmda.go.jp/english/about-pmda/outline/0005.html • PMDA Website: Regulatory Science General Consultation and Regulatory Science Strategy Consultation (R&D) (Japanese) – https://www.pmda.go.jp/review-services/f2f-pre/strategies/0003.html • PMDA Website: Instruction and user fees of Regulatory Science General Consultation and Regulatory Science Strategy Consultation (R&D) (Japanese) – https://www.pmda.go.jp/review-services/f2f-pre/strategies/0006.html – https://www.pmda.go.jp/review-services/f2f-pre/strategies/0005.html
Description	In order to achieve realization of innovative drugs, medical devices, and regenerative medical products originating from Japan, PMDA launched the Pharmaceutical Affairs Consultation on R&D Strategy in July 2011, mainly for universities, research institutions, and venture companies that possess promising "seed-stage" research or technologies. In such consultations, advice will be provided on the tests needed in the early product development stage and the necessary clinical trials. The Pharmaceutical Affairs Consultation on R&D Strategy was turned into Regulatory Science General Consultation and Regulatory Science Strategy Consultation (R&D) in 2017.

ITEM	DESCRIPTION
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Category	Regulatory Building Block
Geographical scope	Japan
Availability	Applicants developing Drug/Medical device/Regenerative Medical Product for rare and non-rare diseases
Scope of use	● Regulatory Science General Consultation Instruction of how to use the Regulatory Science Strategy Consultation (R&D) for developers that are not familiar with medical product development. ● Regulatory Science Strategy Consultation (R&D) 1. Consultation on R&D strategy for drugs/medical devices/regenerative medical products PMDA provides scientific advice in early stage of medical product development (the stage of proof-of-concept study) 2. Consultation on quality and safety for regenerative medical products PMDA provides advice for quality and safety to prepare application of clinical trial 3. Consultation on R&D strategy for pharmaceutical development plans PMDA provides advice for general consideration of clinical trial planning and development plan
Stakeholders	• Drug/Medical device/Regenerative Medical Product developers

ITEM	DESCRIPTION
	• Pharmaceuticals and Medical Devices Agency (PMDA) • Venture companies • Research institutes • Universities
Enablers/ Requirements	N/A
Output	Scientific advice with meeting minutes from PMDA
Best time to apply and time window	• From early stage of medical product development to the stage of proof-of-concept study for consultation on R&D strategy for drugs/medical devices/regenerative medical products • Before a clinical trial notification for consultation on quality and safety for regenerative medical products
Expert tips	Main target of this service is proof-of-concept study or before 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