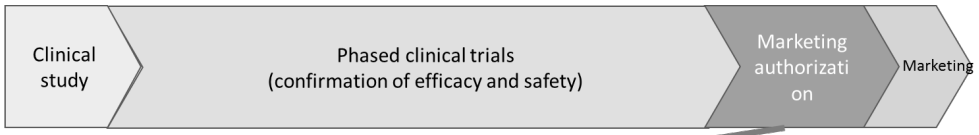
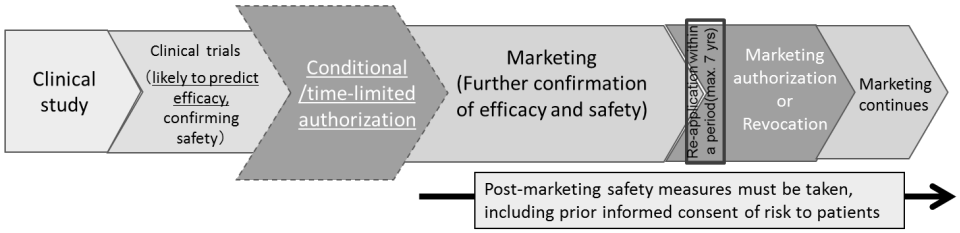
Orphan Drug Development Guidebook

Building Block J308

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 and Time-limited Authorization of Regenerative Medical Products
References	• International Regulatory Forum of Human Cell Therapy and Gene Therapy Products (Japanese) – https://www.pmda.go.jp/review-services/symposia/0050.html • New Japanese initiatives on stem cell therapies. – https://www.ncbi.nlm.nih.gov/pubmed/25842974 • PMDA Asia Training Center Learning Videos: Review 9. Expedited Regulatory Pathways in Japan – https://www.pmda.go.jp/english/int-activities/training-center/0005.html • PMDA website: User Fees (Japanese) – https://www.pmda.go.jp/review-services/drug-reviews/user-fees/0001.html
Description	A regulatory framework for the approval of regenerative medical products to benefit the patients with unmet medical needs under The Law on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices (PMD Act)

ITEM	DESCRIPTION
	[Traditional approval process]  **[New scheme] (for regenerative medical products)**  Fees of some review categories for a regenerative medical product might ne applied.
Category	Regulatory Building Block
Geographical scope	Japan
Availability	Regenerative medicine products developers that have marketing licenses within Japan
Scope of use	To provide patients with early access of regenerative medical products for unmet medical needs
Stakeholders	• Ministry of Health, Labour and Welfare (MHLW) • Pharmaceuticals and Medical Devices Agency (PMDA) • Regenerative medical product developers
Enablers/ Requirements	To predict reasonable likelihood of clinical benefit
Output	Conditional and Time-limited Authorization

ITEM	DESCRIPTION
Best time to apply and time window	After exploratory clinical trials
Expert tips	Recommend to use PMDA consultation or Regulatory Science General Consultation and Regulatory Science Strategy Consultation (R&D) PROs: The Conditional and Time-limited Authorization of Regenerative Medical Products provides patients with early access of regenerative medical products in need CONs: N/A