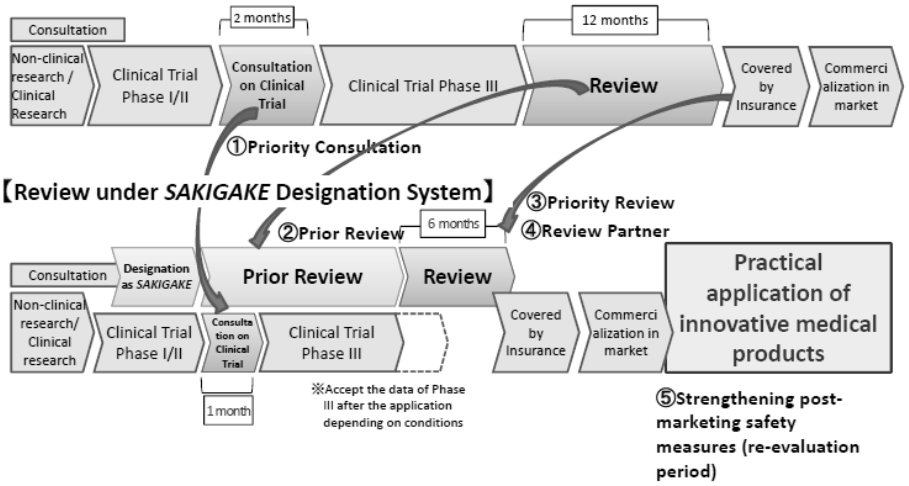
Orphan Drug Development Guidebook

Building Block J302

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	SAKIGAKE Designation System
References	• MHLW Website: Strategy of SAKIGAKE – https://www.mhlw.go.jp/english/policy/health-medical/pharmaceuticals/140729-01.html • MHLW Website: SAKIGAKE Designation System (Japanese) – https://www.mhlw.go.jp/stf/seisakunitsuite/bunya/kenkou_iryou/iyakuhin/tp150514-01_00001.html – https://www.mhlw.go.jp/stf/seisakunitsuite/bunya/000013196.html • 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
Description	SAKIGAKE was introduced based on key measures set forth in the "Strategy of SAKIGAKE", announced by the Ministry of Health, Labour and Welfare (MHLW) in June 2014,and established as legislation in 2019. SAKIGAKE aims at shortening premarket review period for innovative new medical products that satisfy certain criteria, such as severity of intended indication, by designating such products during the early stages of development, and providing prioritized consultation services and premarket pharmaceutical affairs review. By taking advantage of the benefits offered by SAKIGAKE, the target review period for the designated products will be reduced to as short as 6 months, half the standard review period of 12 months for typical new pharmaceutical products.

ITEM	DESCRIPTION
	【Ordinal Review】  1.
Category	Regulatory Building Block
Geographical scope	Japan
Availability	Drugs/medical devices/regenerative medical products developers within Japan that specifically target unmet medical needs. There are four criteria for designation. One of them includes serious or life-threatening medical condition that is often seen in rare diseases.
Scope of use	Producing innovative pharmaceutical products ahead of other countries with the shortest possible development and review process should be aimed through the designation system in which the following measures shall be used: 1) consistent prioritized consultation by the Pharmaceuticals and Medical Devices Agency (PMDA), 2) pre-application consultation in which de facto review is started with data that can be submitted before the application for approval, 3) prioritized review aiming for a further reduction in the total review period, 4) Assigning a manager as a concierge to take on overall management for the whole process toward approval including conformity assurance, quality management, safety measures, and review, 5) strengthening post-marketing safety measures including the extension of the reexamination period.
Stakeholders	• Drug/Medical Devices/Regenerative Medical Products developers

ITEM	DESCRIPTION
	• Ministry of Health, Labour and Welfare (MHLW) • Pharmaceuticals and Medical Devices Agency (PMDA)
Enablers/ Requirements	MHLW may designate SAKIGAKE if the following criteria are fulfilled: 1. Innovativeness of the product: In principle, the product should have a novel mechanism of action that is different from those of approved drug. 2. Treatment for which the earliest commercialization is required for target diseases: The target medical condition should be one of the following: i) Serious or life-threatening medical condition; or 2) Medical condition with persistent symptoms (conditions interfering with normal activities of daily living) for which there is no other curative treatment 3. Highly effective treatment against the target medical condition 4. Develop the product rapidly and file an application for approval in Japan, ahead of other countries
Output	• Prioritized Consultation • Substantial Pre-application Consultation • Priority Review • Review Partner • Substantial Post-Marketing Safety Measures
Best time to apply and time window	Consultation in non-clinical or clinical research stage. Application for designation in early stages of clinical development
Expert tips	PROs: • Prioritized Consultation • Substantial Pre-application Consultation

ITEM	DESCRIPTION
	• Priority Review • Review Partner • Substantial Post-Marketing Safety Measures • Premium at drug pricing CONS: N/A