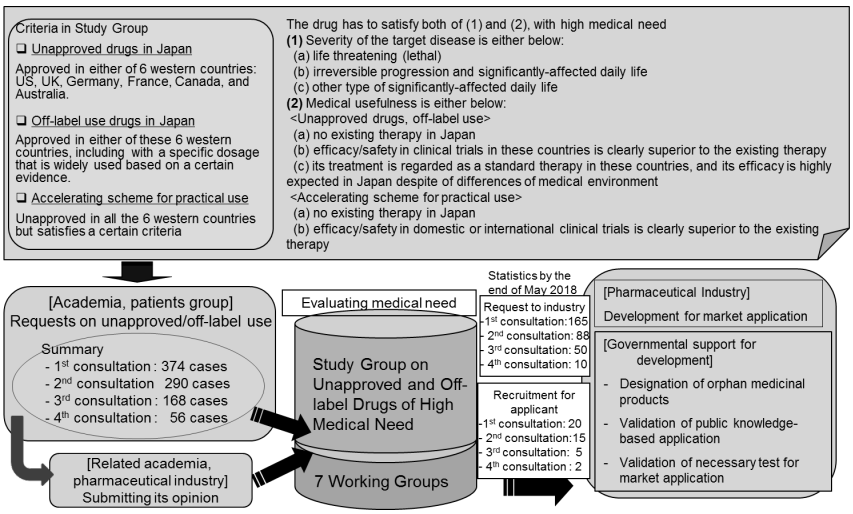
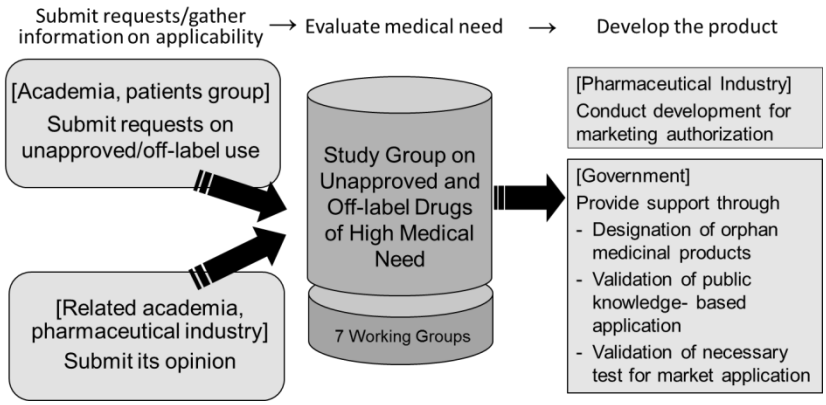
Orphan Drug Development Guidebook

Building Block J310

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	Study Group on Unapproved and Off-label Drugs of High Medical Need
References	• MHLW's website: Study Group on Unapproved and Off-label Drugs of High Medical Need (Japanese) ○ https://www.mhlw.go.jp/stf/shingi/other-iyaku_128701.html
Description	The objective of the Study Group is to evaluate medical need, investigate necessary studies for market approval, and request company to develop medicinal products to solve the problem of unapproved drug and off-label use with medical need. Public consultations are conducted to gather requests from public.  Process Flow

ITEM	DESCRIPTION
	
Category	Regulatory Building Block
Geographical scope	Japan
Availability	Applicants developing medicines for rare and non-rare diseases
Scope of use	The scope of this initiative is to evaluate medical need, investigate necessary studies, and promote drug developments for market approval to solve the problem of unapproved drug and off-label use with medical need.
Stakeholders	• Patient Group • Academia (Academic Societies) • Individuals • Drugs developers • Study Group (Physicians, Pharmacists, and other medical professions)

Enablers/ Requirements	Applicable criteria
	• Unapproved drugs in Japan
	  ○ Approved in either of 6 countries (US, UK, Germany, France, Canada, and Australia (but not Japan))
	• Off-label use drugs in Japan (Approved for other indication in Japan)
	  ○ Approved in either of above 6 countries, or

ITEM	DESCRIPTION
	○ widely used in either of above 6 countries with a specific dosage, based on a certain evidence • Accelerating scheme for practical use ○ Unapproved in all 6 countries but satisfies a certain criterion such as the existence of ongoing/completed investigator-initiated phase III trial in Japan or adequate evidence from clinical study.
Output	Request of drug development and various support to company
Best time to apply and time window	At the time of public consultation conducted
Expert tips	For more information, please refer to information in Japanese. PROs: Possible Incentives currently applicable to development on Unapproved Drugs or Off-label Drugs • Priority Review • Orphan Drug Designation • Conditional Early Approval System for Drugs