Orphan Drug Development Guidebook

Building Block J314

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	Japan Agency for Medical Research and Development (AMED) Clinical Innovation Network – Diseases-related Registries
References	https://www.mhlw.go.jp/file/05-Shingikai-10801000-Iseikyoku-Soumuka/0000204815.pdf https://www.jstage.jst.go.jp/article/jniph/67/2/67_191/_pdf/-char/ja https://www.amed.go.jp/en/program/list/05/01/015.html https://cinc.ncgm.go.jp/
Description	Patient registries are expected to play an important role in enhancing clinical trials and studies including patient allocation, recruitment of enrollment, as well as serving as control arm for clinical trials especially in rare diseases and post marketing surveillance. However, many registries are operated by different healthcare bodies for various different purposes and are not originally designed to collect information relevant to clinical trials. Therefore, it is currently difficult for researchers and pharmaceutical companies to identify each registries and utilize them for research and development purposes. Project for Promoting Clinical Innovation Network (CIN) aims to promote the use of patient registries in clinical development and studies. For this purpose, the project is gathering information on registry databases around Japan and organize them by their intended purpose, and create a one stop service that enables users to search for the registry database of their will from one single search engine. The ultimate goal is to provide patients, researchers and industries with updated information on the CIN for further clinical use and medical development.

ITEM	DESCRIPTION
Category	Development Practices Building Blocks
Geographical scope	Japan
Availability	For applicants developing medicines and medical devices for rare diseases.
Scope of use	A) Market surveillance: The number of patients registered and their geographical location could be used to estimate feasibility and marketability of developing a certain drug against a disease in Japan. B) Patient recruitment: Patients registered in the database could efficiently be recruited to enroll in a clinical trial of study. C) Control arm of a clinical trial: The natural history of the patients with a certain disease can be collected from the registry, and serve as the control arm of a clinical trial (Single Arm clinical studies）. D) Post-marketing safety surveillance: The registry will enable users to capture any adverse events that developed after the product has been released to the market.
Stakeholders	• Drug/Medical device developers • Registry owners
Enablers/ Requirements	No requirement
Output	The end product of the clinical innovation network (CIN) is expected to play a major role in clinical development and post-marketing surveillance of rare diseases where a large scale, conventional type clinical studies are difficult to perform
Best time to apply and time window	Anytime during the drug development
Expert tips	PROs:

ITEM	DESCRIPTION
	• Reduction of medical development costs • Reduced time necessary for clinical development CONs: N/A