

Orphan Drug Development Guidebook

Building Block J309

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	Designated Intractable/Rare Diseases (Designated Nan-byo)
References	<ul style="list-style-type: none"> • Annual Health, Labour and Welfare Report (MHLW) <ul style="list-style-type: none"> – https://www.mhlw.go.jp/english/wp/index.html • The measures for intractable/rare diseases (Japanese) <ul style="list-style-type: none"> – http://www.nanbyou.or.jp/entry/3756 <p>Additional Information</p> <ul style="list-style-type: none"> • National Registry of Designated Intractable Diseases in Japan: Present Status and Future Prospects <ul style="list-style-type: none"> – https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5243159/
Description	The system of designated intractable/rare diseases (Designated Nan-byo) is a unique system in Japan. Definition of designated Nan-byo is provided in the law named "Act on Medical Care for Patients with Intractable/Rare Diseases". Patients with designated Nan-byo can receive financial support for medical treatment.
Category	Regulatory Building Block
Geographical scope	Japan
Availability	Applicants developing medicines for rare diseases.
Scope of use	Designated Nan-byo is used as a requirement to fulfill the criterion of "Number of Patients" in Orphan drugs/medical devices/regenerative medical products designation in Japan.

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Stakeholders	<ul style="list-style-type: none"> ● Patients ● Ministry of Health, Labour and Welfare (MHLW)
Enablers/ Requirements	<p>Following criteria need to be fulfilled</p> <ol style="list-style-type: none"> 1. The cause of the disease is not clear 2. The treatment method is not established 3. A rare disease 4. A long-term cure is needed 5. Patient's ratio does not reach 0.1% of the whole population 6. Subjective diagnostic criteria (or equivalent with diagnostic criteria) for a given disease is established
Output	<ul style="list-style-type: none"> ● Promotion of survey/research ● Establishment of medical institutions ● Reduction of co-payment of medical fees ● Improved/enhanced cooperation in community-based health/medical care and welfare ● Promotion of welfare measures aimed at improving QOL
Best time to apply and time window	N/A
Expert tips	<p>Detailed information of each designated Nan-byo is available in Japanese at http://www.nanbyou.or.jp/</p> <p>PROs:</p>

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	<p>Designated Nan-byo is used as a requirement to fulfill the criterion of "Number of Patients" in Orphan drugs/medical devices/regenerative medical products designation in Japan.</p> <p>CONs:</p> <p>N/A</p>