The 22nd DIA Japan Annual Meeting 2025

Strong Ties of Japan with Asia and the World for Delivering
‘Tomorrow's Normal’

October 19-21, 2025 | Tokyo Big Sight

S35
Health Equity: Tomorrow’s Normal in Rare Diseases
希少疾患領域におけるHealth Equityの明日のあたりまえ

Hiroto Aso, PMDA

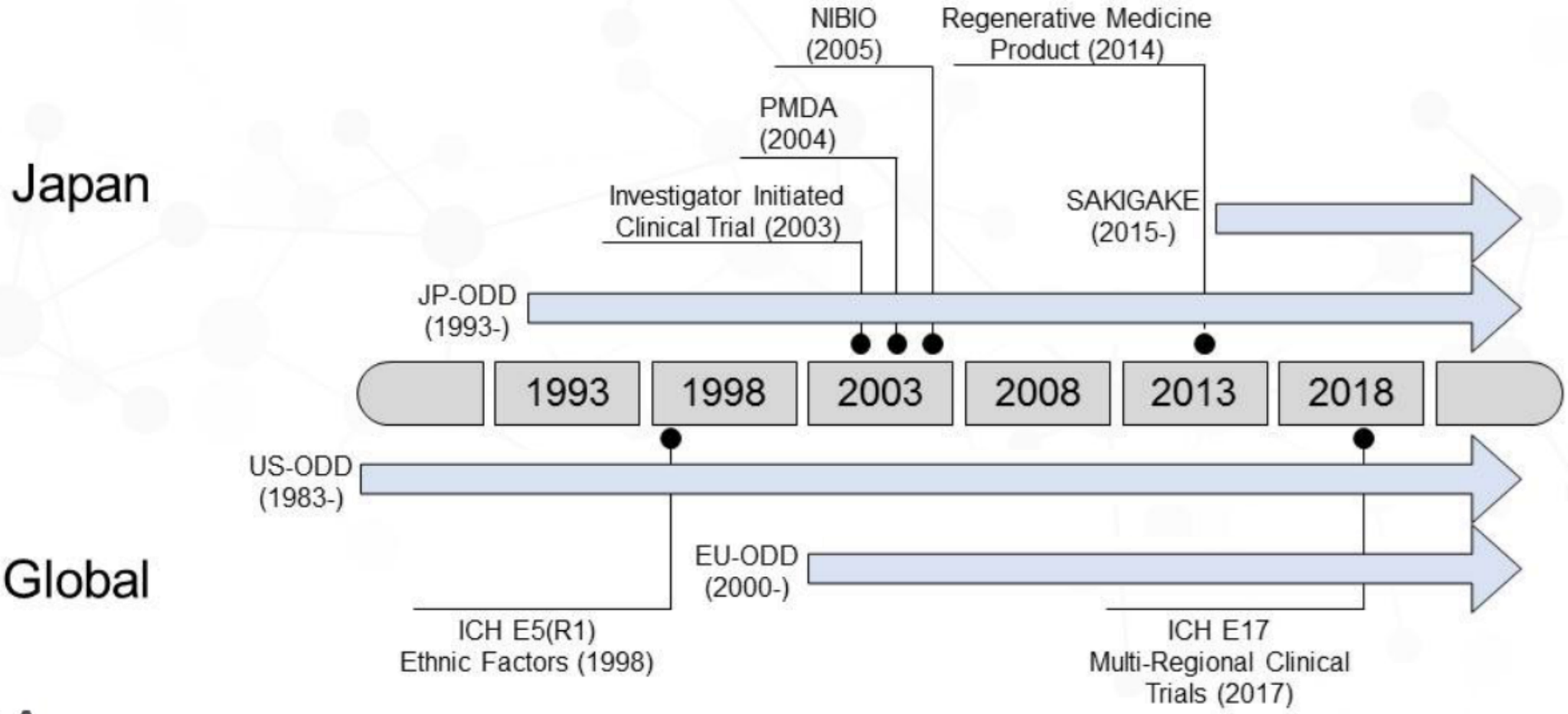
DIA

Disclaimer

The views and opinions expressed in the following PowerPoint slides are those of the individual presenter and should not be attributed to DIA, its directors, officers, employees, volunteers, members, chapters, councils, Communities or affiliates.

This presentation is incomplete without accompanying verbal commentary.

A timeline of orphan-drug-related events and milestones in Japan and worldwide



Outline - Orphan Drugs Designation system in Japan

Aim

✓ To promote R&D on products for rare diseases, aiming to provide the people with the safe and effective medicines/medical devices as early as possible

Designation Criteria

1. Number of patients (that any of the followings is satisfied)
   - Less than 50,000* in Japan
   - The target disease is designated as “Nan-byo”.
2. Medical needs
   - For serious diseases with high medical needs
3. Possibility of development

*Equivalent to 0.04% of the Japanese population

**National Institutes of Biomedical Innovation, Health and Nutrition

Incentives

- Grant-in-Aid for R&D on orphan designated drugs (NIBN**)
- Tax deduction for R&D expenses
- Priority scientific consultation (PMDA)
- Priority review (PMDA)
- Premium at drug pricing
- Extension of re-examination period***

Promoting R&D

***Market exclusivity period

Trends in orphan drug designation and approval

Japan	US	EU
*Meet **all** of the following* • *Patient population* (less than 50,000 in Japan, or the disease has to be designated as Nan-byo) • *Serious diseases with high medical needs* • *Possibility of development*	*Meet **any** of the following* • *Less than 200,000 patients in the United States* • *It is difficult to recoup development costs in the United States*	*Meet **all** of the following* • *Patient population (5 patients per 10,000 people)* • *Serious disease* • *Medical needs*

Designation Designation that received approval for marketing

※Source：https://www.accessdata.fda.gov/scripts/opdlisting/oopd/index.cfm（September, 2025）
https://www.ema.europa.eu/en/human-regulatory/overview/orphan-designation-overview（September, 2025）

Revisions of orphan drug designation criteria in Japan

PSB/PED Notification No. 0116-1
PSB/MDED Notification No. 0116-1
January 16, 2024

To: Prefectural Health Departments (Bureaus)

From: Director, Pharmaceutical Evaluation Division,
Pharmaceutical Safety Bureau,
Ministry of Health, Labour and Welfare
(Official seal omitted)

Director, Medical Device Evaluation Division,
Pharmaceutical Safety Bureau,
Ministry of Health, Labour and Welfare
(Official seal omitted)

Partial Revision of "Designation of Orphan Drugs etc."

Designation of orphan drugs, orphan medical devices, and orphan regenerative medicinal products (hereinafter referred to as "orphan drugs etc.") based on Article 77-2 (1) of the Act on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices (Act No. 145 of 1960, hereinafter referred to as "the Act") has been made in accordance with "Designation of Orphan Drugs etc." (Joint PSEHB/PED Notification No. 0831-7 issued by the Director, Pharmaceutical Evaluation Division and PSEHB/MDED Notification No. 0831-7 issued by the Director of Medical Device Evaluation Division, Pharmaceutical Safety and Environmental Health Bureau, Ministry of Health, Labour and Welfare, dated August 31, 2020, hereinafter referred to as the "Notification by Directors").

Administrative Notice
January 16, 2024

To: Pharmaceutical Affairs Section, Prefectural Health Department (Bureau)

Pharmaceutical Evaluation Division,
Pharmaceutical Safety Bureau,
Ministry of Health, Labour and Welfare

Questions and Answers (Q&A) for Designation of Orphan Drugs etc.

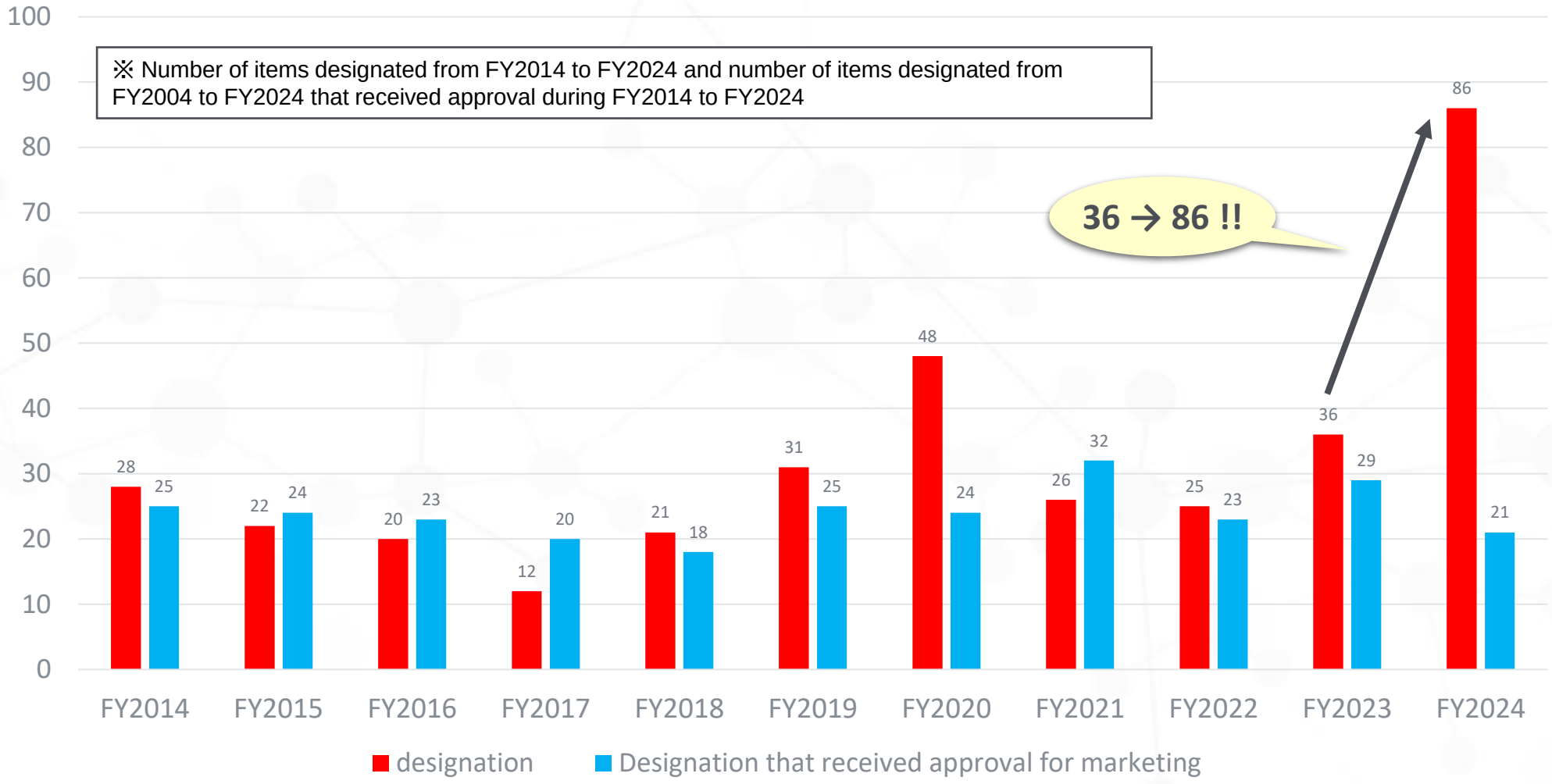
Handling of the designation of orphan drugs has been shown in "Designation of Orphan Drugs etc." (Joint PSEHB/PED Notification No. 0831-7 issued by the Director, Pharmaceutical Evaluation Division and PSEHB/MDED Notification No. 0831-7 issued by the Director of Medical Device Evaluation Division, Pharmaceutical Safety and Environmental Health Bureau, Ministry of Health, Labour and Welfare, dated August 31, 2020, hereinafter referred to as the "Notification by Directors")

The notification and Q&A document were issued in January 2024.
https://www.pmda.go.jp/english/rs-sb-std/rs/0008.html

Revisions of orphan drug designation criteria in Japan

	Old	New
Patient population	● The number of subjects pertaining to the usage of the drugs is less than 50,000 in Japan. (In principle, so-called “salamislicing”(=orphan subset without any clear medical or pharmaceutical reasons) is NOT acceptable.) ● For a designated intractable disease, less than the number of subjects specified in the Designated Intractable Disease Act	➔Expanded the range of orphan subset considered acceptable
Medical needs	● For serious diseases ● High medical needs that meet either of the following: ✓ No appropriate alternative drugs etc. or treatments ✓ Expected to have significantly higher efficacy and safety compared to existing drugs etc.	➔Clarified and added some examples
Possibility of development	● There are rationales for the use of the drugs for the target disease ● Its development plan is recognized to be appropriate	➔Clarified

Trends in orphan drug designation and approval in Japan



Current status of drug loss

- As of March 2023, there are **143 pharmaceutical products approved in EU and the US but not yet approved in Japan (unapproved drugs).**
- Of the 143 unapproved drugs, **86 items (60.1%) have not yet entered domestic development.** This indicates that drug lag / loss is occurring, where approval application are not even submitted in the first place (i.e., companies are not developing them.)
- An analysis of trends among the 86 drug items for which domestic development has not yet commenced revealed that a **relatively large proportion are venture-originating drugs, orphan drugs, or drugs for pediatric use.**

	approved	unapproved	developping or not in unapproved items	
			developping	**not yet developping**
US	136	7	3	4
EU	86	57	26	31
Japan	0	**143**	57	**86 items**

breakdown

venture originating	**orphan drug**	pediatric use
56% (48 items)	**47%** (40 items)	**37%** (32 items)

※Of the 86 items in the list, 14 items (16%) are neither venture, orphan, nor pediatric products.

※Source : https://www.mhlw.go.jp/content/11121000/001484839.pdf (Japanese only)

Government initiative against drug loss

ひと、くらし、みらいのために
厚生労働省
Ministry of Health, Labour and Welfare

Press Release

令和７年３月 31 日
【照会先】
医政局研究開発政策課治験推進室
室長　　飯村　康夫（内線 4161）
室長補佐　酒井　義瑛（内線 4165）
（代表電話）03(5253)1111
（直通電話）03(3595)2430

報道関係者　各位

令和６年度厚生労働科学特別研究事業
「ドラッグ・ロスの実態調査と解決手段の構築」研究班の
整理結果を公表します

この度、令和６年度厚生労働科学特別研究事業「ドラッグ・ロスの実態調査と解決手段の構築」（研究代表者：国立がん研究センター中央病院先端医療科 佐藤 潤 医員）における、欧米では承認されているが国内では承認されていない医薬品のうち国内開発未着手の医薬品（以下「ドラッグ・ロス品目」という。）の情報の整理結果が別添のとおり取りまとめられましたので公表します。

厚生労働省では、この整理結果を踏まえ、ドラッグ・ロスの更なる解消に向けて、「開発の必要性が特に高い医薬品」（グループA品目：14 品目）について、医療上の必要性の高い未承認薬・適応外薬検討会議を開催する予定です。同会議において、医療上の必要性が高いと評価された品目については、国内企業への開発要請等を行い、現在生じているドラッグ・ロスの解消に取り組んでまいります。

また、「開発の必要性が高い医薬品」（グループB品目：41 品目）については、「未承認薬等迅速解消促進調査事業」において、医療上の必要性の高い未承認薬・適応外薬検討会議における評価に必要な情報の整理を行い、準備ができたものから順次、医療上の必要性の高い未承認薬・適応外薬検討会議で医療上の必要性を評価することとしています。

【特別研究班による分類結果】

グループA「開発の必要性が特に高い医薬品」	：14 品目
グループB「開発の必要性が高い医薬品」	：41 品目
グループC「開発の必要性が低い医薬品」	：11 品目
グループD「開発の必要性がない医薬品」	：12 品目
その他「既にドラッグ・ロスが解消されている医薬品」	： 8 品目

Regarding 86 pharmaceutical items not yet under domestic development, the government proactively compiled the information necessary for evaluating medical needs.

↓

The items are evaluated to whether to request development in the Unapproved Drugs and Off-Label Use Review Committee.

↓

Development request !!

- ARTESUNATE (malaria)
- AYVAKIT (Gastrointestinal Stromal Tumor)
- AKLIEF (Acne vulgaris)
- XENLETA (Bacterial pneumonia)
- NUZYRA (Bacterial pneumonia, Acute bacterial skin/skin structure infection)
- ANTHIM (anthrax)
- PRETOMANID (Multi-drug-resistant tuberculosis)
- OMEGAVEN (Parenteral Nutrition-Associated Cholestasis)

※red ink items are designated orphan drug in US or EU

※Source: https://www.mhlw.go.jp/stf/shingi2/0000198856_00040.html (Japanese only)

Thank you for your attention