20th DIA Japan Annual Meeting 2023

"Rebuilding Drug Development through Society 5.0:
The Fusion of Knowledge and Technology that Transcends Time and Space"

November 5-7, 2023 | Ariake Central Tower Hall ＆ Conference

SPECIAL SESSION 1
Orphan Drug Development Update: Issues and Measures for Global Cooperation

Regulatory approach to promote orphan drug development in Japan

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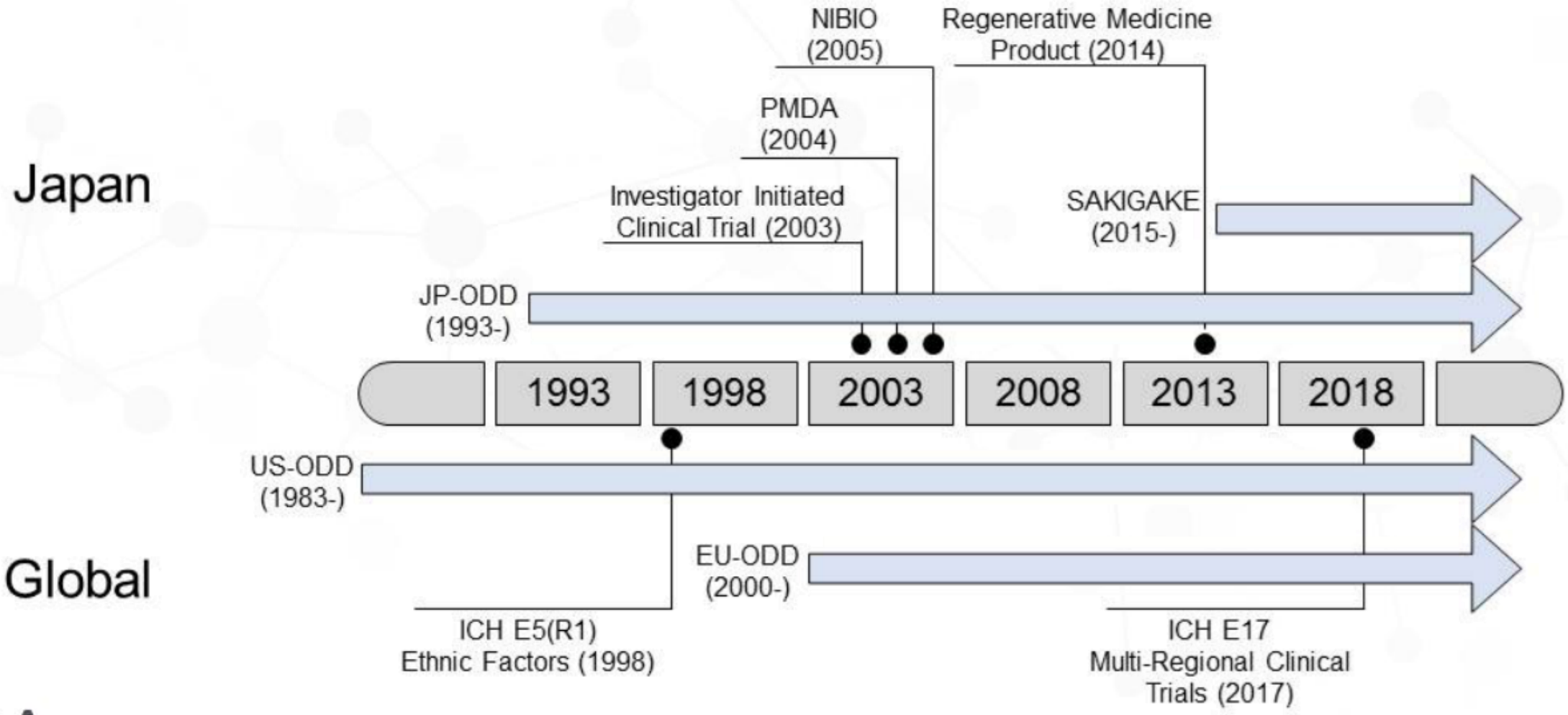
A timeline of orphan-drug-related events and milestones in Japan and worldwide



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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 one of the designated intractable disease

*Equivalent to 0.04% of the Japanese population

2. Medical needs
   - For serious diseases with high medical needs
3. Possibility of development

Incentives

**National Institutes of Biomedical Innovation, Health and Nutrition

- Grant-in-Aid for R&D on orphan designated drugs (NIBIOHN**)
- 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

Legal basis of orphan drug designation

Legislation etc.	Corresponding part	Description
PMD Act[1)]	Article 77-2	Overview of orphan drug designation system
Regulation for Enforcement of PMD Act[2)]	Article 251	Upper Limit on the Number of Patients
PED/MDED Notification No.831-7[3)]	All	Details of designation criteria

1) Act on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices (https://www.japaneselawtranslation.go.jp/ja/laws/view/3213/en)
2) Regulation of Enforcement of the Act on Securing Quality, Efficacy and Safety of Products Including Pharmaceuticals and Medical Devices (https://www.japaneselawtranslation.go.jp/ja/laws/view/3215/en)
3) https://www.mhlw.go.jp/web/t_doc?dataId=00tc5284&dataType=1&pageNo=1 (Japanese only)

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Criteria for orphan drug designation in Japan

Patient population

- The number of patients who may use the drugs, medical device or regenerative medicine should be less than 50,000 in Japan, or the disease has to be designated as Nan-byo (intractable and rare disease).
- Intractable and rare diseases are designated as Nan-byo by the MHLW based on the Japanese Act on Medical Care for Patients with Intractable/Rare Diseases.

Medical needs

- The drugs, medical devices or regenerative medicine should be indicated for the treatment of serious diseases, including difficult-to-treat diseases. In addition, they must be drugs, medical devices or regenerative medicine for which there are high medical needs satisfying one of the following criteria.
  - ✓ There is no appropriate alternative drug/medical device/regenerative medicine or treatment
  - ✓ High efficacy or safety is expected compared with existing products

Possibility of development

- There should be a theoretical rationale for the use of the product for the target disease, and the development plan should be appropriate.

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Trends in orphan drug designation and approval in Japan

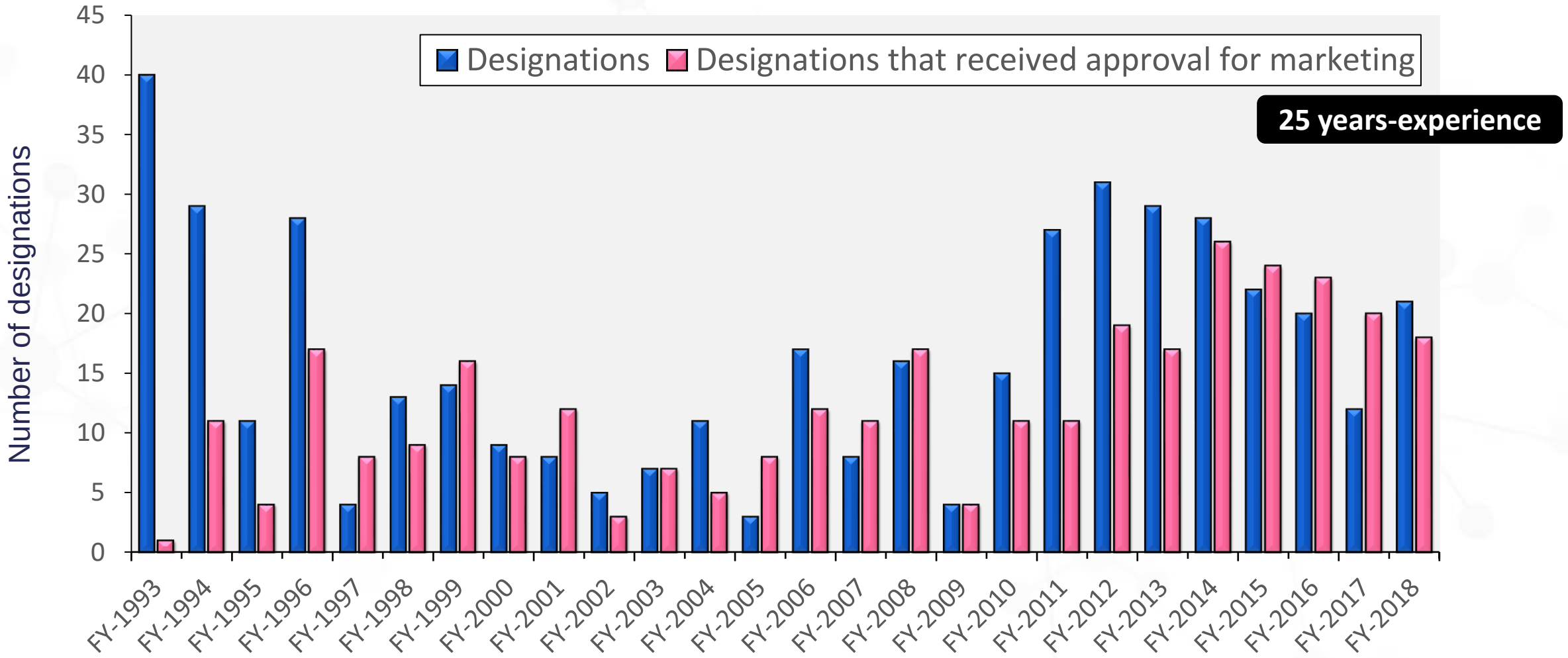

Fig.1 **Trends in orphan drug designation and approval in Japan.** Annual number of orphan drug designations and approvals for marketing from fiscal year (FY) 1993 to FY 2018. The same data shown in Nat Rev Drug Discov. 2021; 20: 893-4.

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Distribution of prevalence and disease category for orphan drug designations in Japan

25 years-experience

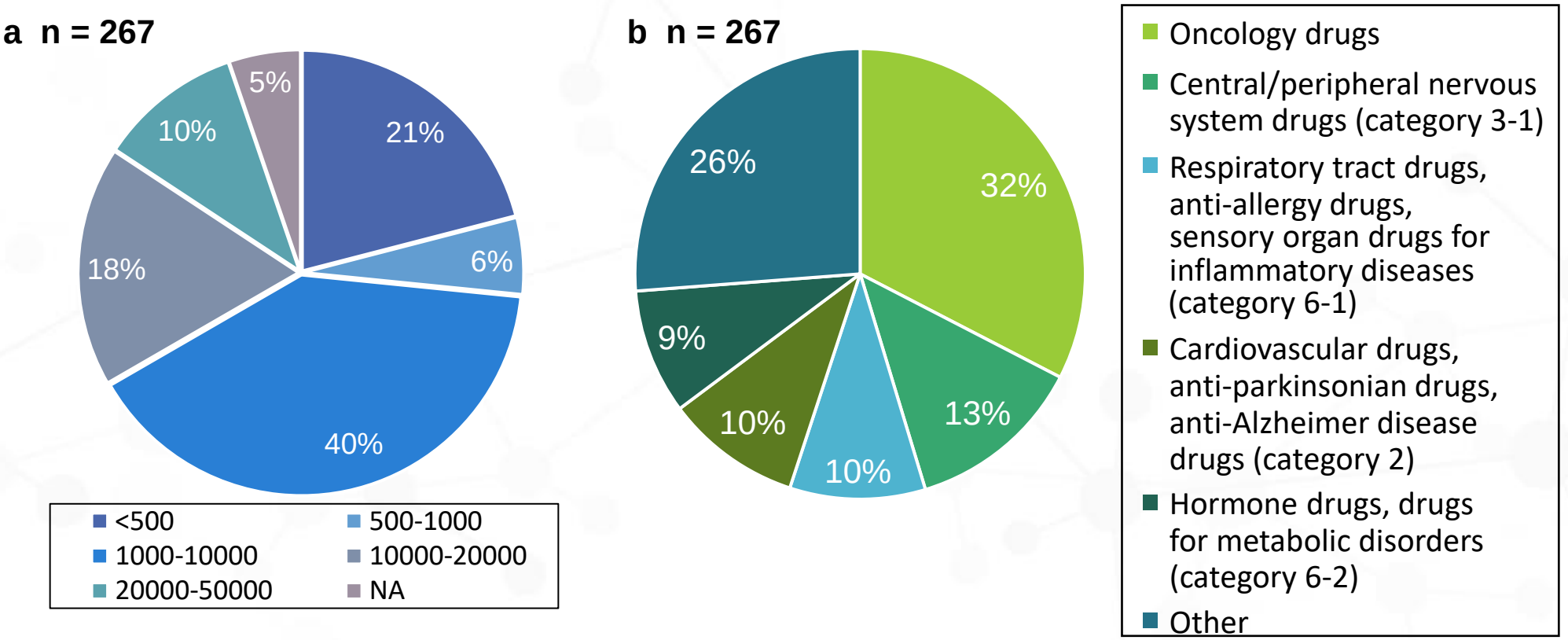

Fig.2 | **Distribution of prevalence and disease category for orphan drug designations in Japan. a** | Distribution of the estimated number of patients for indications for which products received orphan drug designation from fiscal year (FY) 2004 to FY 2018. NA, not available, includes vaccine products for pandemic H5N1 influenza. **b** | Distribution of orphan drug designations from FY 2004 to FY 2018 by disease categories currently used in the regulation in Japan. The same data shown in Nat Rev Drug Discov. 2021; 20: 893-4.

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Criteria for orphan drug designation in Japan to be revised soon

希少疾病用医薬品の指定のあり方について

第１回　創薬力の強化・安定供給の確保等のための薬事規制のあり方に関する検討会

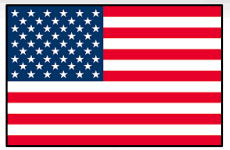
ELSEVIER

Drug Discovery Today

Volume 28, Issue 10, October 2023, 103755

Feature

Increasing orphan drug loss in Japan: Trends and R&D strategy for rare diseases

			
Period	(FY)1993-2022	1983-2022	2000-2022
Designation	562	6,352	2,734
Approval	426	1,108	231
% of Approval	76%	17%	8%
Reference	• https://www.nibiohn.go.jp/nibio/part/promote/files/ph_orphanlist_drug_JP_231003.pdf • https://www.accessdata.fda.gov/scripts/opdlisting/oopd/ • https://www.ema.europa.eu/documents/report/annual-report-use-special-contribution-orphan-medicinal-products-2022_en.pdf		

Based on MHLW committee discussions, criteria for orphan drug designation will be revised soon to deal with increasing orphan drug loss in Japan.

For earlier and more designations

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International collaboration for orphan drug development

- In March 2014, Joint EMA/FDA/MHLW-PMDA workshop was held to provide information of support for orphan drug development.

https://www.ema.europa.eu/en/events/joint-european-medicines-agency-ema-us-food-drug-administration-fda-japanese-ministry-health-labour


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Joint European Medicines Agency (EMA), US Food and Drug Administration (FDA), and Japanese Ministry of Health, Labour and Welfare (MHLW) and Pharmaceuticals and Medical Devices Agency (PMDA) orphan medicinal product workshop

Share

Date: 10/03/2014

Location: European Medicines Agency, London, UK

The aim of the workshop is to provide information to companies, as well as to academics, on the EMA, FDA and MHLW-PMDA systems for orphan medicinal product designation as well as on the grant programmes for development of orphan medicinal products that are available. These programmes aim to promote the development of new medicines for the treatment of rare diseases. This workshop is now fully booked. However, the plenary session will be publicly broadcasted on the EMA website (prior registration not required) - please click on the 'multimedia' tab to watch live on the day. Following the workshop, the recording and presentation slides will also be made available.

- In 2016, EMA, FDA, and MHLW-PMDA reported a paper of the worldwide collaboration for orphan drug designation

Nature reviews Drug discovery. 2016;15:440-441


CORRESPONDENCE

Worldwide collaboration for orphan drug designation

Segundo Mariz, James H. Reese, Kerstin Westermark, Lesley Greene, Takahiro Goto, Tatsuro Hoshino, Jordi Llinares-Garcia and Bruno Sepodes

Recent international communication for promoting orphan drug development

Date	Title	Meeting
Jun. 2023	Regulatory approach to promote orphan drug development in Japan	DIA China Annual Meeting 2023
Oct.2021	Regulatory approach to promote orphan drug development in Japan	9th Joint Conference of Taiwan and Japan on Medical Products Regulation
Feb.2020	Clinical trials and regulatory supports for innovative drug development in Japan	4th India-Japan Medical Products Regulation symposium
Oct.2019	Accelerating orphan/innovative drug development in Japan - Current status & Challenges	2019 Global Health Forum in Taiwan

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Questions?

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