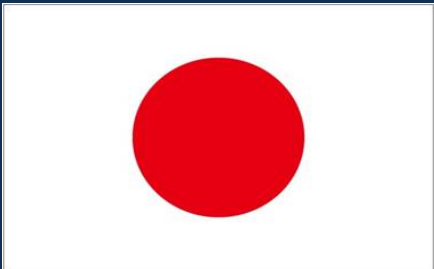


# OVERVIEW OF ORPHAN DRUG DESIGNATION (ODD) PROCESS IN JAPAN

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**GLOBAL**  
REGULATORY PARTNERS

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# Definition of ODD in Japan

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An “Orphan Drug” is defined as pharmaceuticals designated as such by the Minister of Health, Labour, and Welfare (“MHLW”) under the Act on Securing Quality, Efficacy, and Safety of Products Including Pharmaceuticals and Medical Devices. More specifically, MHLW can designate a pharmaceutical as an Orphan Drug when all of the following requirements are satisfied: (i) the number of patients who need the drug is less than 50,000; (ii) there is no alternative drug and the drug has particularly excellent efficacy or safety; and (iii) the manufacturers have a possible and reasonable development plan for the drug. With regard to requirement (i), as an exception, if a disease falls under a category provided in the Intractable Disease Act, the number of the patients may defer to the provision of the said Act.

**The Orphan Drug Designation is approved by MHLW based on the positive opinion of the Pharmaceutical Affairs and Food Sanitation Council (PAFSC).**

# Institutions Involved in the ODD Process in Japan

**Ministry of the Health,  
Labor and Welfare  
(MHLW)**



- Review and designation of orphan drugs/medical devices
- Review and approval of orphan drugs/medical devices
- Pre-designation consultation for orphan drugs/medical devices
- Payment for the operational cost of NIBIO

**Pharmaceuticals and  
Medical Devices Agency  
(PMDA)**



- Priority scientific consultation for clinical trials and dossiers for marketing authorization of orphan drugs/medical devices.

**National Institute of  
Biomedical Innovation  
(NIBIO)**



- Subsidy payment to the applicant
- Accreditation for research expenses to be used by the applicant
- Provision of guidance and consultation to the applicant

# Qualification Criteria for ODD in Japan

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To qualify for an ODD (Orphan Drug Designation), a drug should satisfy the following criteria:

## 1 Number of Patients

- The number of patients who may use the drug should be **less than 50 000 in Japan**.
- The number of patients could be estimated based on the report of Health and Labor Science Research or the data published by reliable scientific societies.
- Submission of an estimate based on multiple statistical methods is recommended.

## 2 Medical Needs

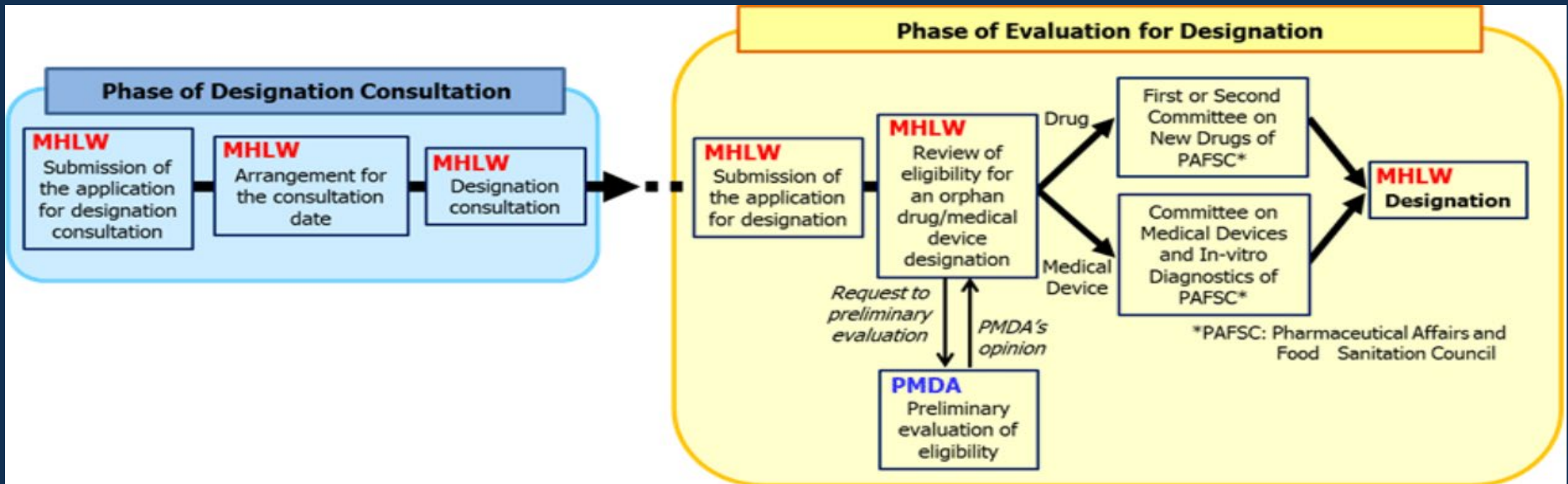
- The drugs should be indicated for the treatment of serious diseases, including difficult-to-treat diseases.
- They must be drugs for which there are high medical needs satisfying one of the following criteria:
  - (1) There is no appropriate alternative drug or treatment;
  - (2) High efficacy or safety is expected compared with existing products.

## 3 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.

# Process of Orphan Drug Designation (ODD) in Japan

- The application for an orphan drug designation can be submitted at any time to MHLW during the drug development process.
- The ODD process includes 2 phases: Phase of Designation Consultation and Phase of Evaluation for Designation as shown in Figure 1 here below



**Figure 1:** Outline of Process for Orphan Drug Designation

# Process of Orphan Drug Designation (ODD) in Japan

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## Phase of Designation Consultation

- Applicants should submit the application for ODD consultation to MHLW.
- Consultation should be held **before** applying for ODD.
- Applications should be sent by mail or fax and should be written in Japanese.
- Consultations are limited to 30-minute session in principle.
- The number of consultation attendees should be appropriate for the extent of consultation items.
- The supporting documents should be sent to MHLW 1 week before the consultation day.

# Process of Orphan Drug Designation (ODD) in Japan

## Phase of Evaluation of Application for Designation

- Applicants should submit the application form for ODD with supporting data as attachments

Application Form for Drug	<ul style="list-style-type: none"><li>▪ Number of copies to be submitted: One original and two copies</li><li>▪ Submit to: Evaluation and Licensing Division, Pharmaceutical and Food Safety Bureau, MHLW</li></ul>
Attachments to the application	<ul style="list-style-type: none"><li>▪ Data on the number of patients: Objective statistical data on the number of patients in Japan for whom the drug should be indicated.</li><li>▪ Data on medical needs:<ul style="list-style-type: none"><li>- Data on the diseases such as etiology and symptoms</li><li>- Data on the current status such as availability of similar drugs and treatment</li></ul></li><li>▪ Data on the theoretical rationale for the use of the drugs Related data in a draft dossier of application for marketing authorization, which is available at the time of application for orphan drug</li><li>▪ Development plan (data on the possibility of development), including the current development status, expected test items, duration of the study and necessary expenses.</li><li>▪ A Summary of the Orphan Drug for the Committee meetings and publication</li></ul>

# Government Incentives for ODD in Japan

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## Subsidy Payment

- Orphan drug applicants can receive subsidies through the **National Institute of Biomedical Innovation (NIBIO)** to reduce the financial burden of product development. (The total budget for financial year 2010 was 650 million yen.)

## Preferential Tax Treatment

- Twelve percent of study expenses for orphan drug incurred during the NIBIO subsidy payment period (not including subsidies granted by NIBIO) can be reported as a tax credit.

# Government Incentives for ODD in Japan

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## Guidance and Consultation

- Orphan drug applicants can receive guidance and consultation from the **Ministry of Health, Labor and Welfare**, the **Pharmaceuticals and Medical Devices Agency (PMDA)**, and **NIBIO** on research and development activities.
- PMDA provides a priority consultation system for designated orphan drug.
- Lower user fee categories for PMDA's consultation are applicable to designated orphan drugs.

# Government Incentives for ODD in Japan

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## Priority Review

- Designated orphan drugs are subject to priority review for marketing authorization to ensure that they are supplied to clinical settings at the earliest possible opportunity.
- Categories of lower user fees are applicable to review for marketing authorization of designated orphan drugs.

## Extension of re-examination period

- After orphan drug designation and approval, the re-examination period for the drugs can be extended up to 10 years for drugs and up to 7 years for medical devices.

# REFERENCES

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Ministry of Health, Labor and Welfare Website. Link:

[https://www.mhlw.go.jp/english/policy/health-medical/pharmaceuticals/orphan\\_drug.html#:~:text=In%20Japan%2C%20drugs%20and%20medical,if%20they%20are%20intended%20for](https://www.mhlw.go.jp/english/policy/health-medical/pharmaceuticals/orphan_drug.html#:~:text=In%20Japan%2C%20drugs%20and%20medical,if%20they%20are%20intended%20for)