EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Japanese Orphan Drug Designation

In Japanese (日本語)

Overview of Orphan Drug/Medical Device Designation System

Orphan Drug/Medical Device Designation

In Japan, drugs and medical devices can be designated as orphan drugs or medical devices based on the Article 77-2(PDF:87KB) of the Pharmaceutical Affairs Law if they are intended for use in less than 50 000 patients in Japan and for which there is a high medical need. They are designated by the Minister of Health, Labour and Welfare based on the opinion of the Pharmaceutical Affairs and Food Sanitation Council (PAFSC). Designation of orphan drugs/medical devices does not automatically lead to marketing approval.
The objectives and outline of the system are described below.

Background

Before the orphan drug/medical device system had established, drugs and medical devices to be used for the treatment of difficult-to-treat diseases and acquired immune deficiency syndrome (AIDS) had not been sufficiently developed despite the high medical needs because the number of patients was small. With the diversification of public healthcare needs, safe and quality medical products were required to be supplied to patients as soon as possible. Accordingly, based on rising public expectations and the changing circumstances of drug and medical-device research and development, it had been decided to take special measures to support and promote research activities for the development of orphan drugs/medical devices.

Designation criteria

The Minister of Health, Labour and Welfare may designate drugs and medical devices satisfying the following criteria as orphan drugs/medical devices after receiving applications for orphan designation from the applicants.

(1) Number of patients

The number of patients who may use the drug or medical device should be less than 50 000 in Japan.

- The number of patients could be estimated based on the report of Health and Labour Science Research or the data published by reliable scientific societies. The number of patients with a difficult-to-treat disease is sometimes difficult to estimate accurately due to lack of research on the patient population. Therefore, estimates from a variety of statistical data are generally used to indicate that the number of those patients is less than 50 000 in Japan. Submission of an estimate based on multiple statistical methods is recommended.

Introduction

Medicines and medical devices for patients with rare diseases are clinically very important.

However,

the lack of the attraction for developing these medicinal products due to the small numbers of targeting patients makes it difficult to precede favourable research and development.

So, In order to support the patients with rare diseases,

it is necessary to establish proper measures to promote research and development for orphan medicinal products.

Under these circumstances,

Orphan system was established in Japan by amending the Pharmaceutical Affaires Law in April 1993, and entered into force in 1st October in the same year.

Related legislations & Guidelines

- The Pharmaceutical Affaires Law (PAL)
  - Chapter 9-3: Designation etc. of Orphan Drugs and Orphan Medical Devices
    - Stipulation of overall and general Orphan system
- Enforcement Regulation of the PAL
  - Chapter 9: Designation etc. of Orphan Drugs and Orphan Medical Devices.
    - Criteria, Application form etc.
- Notification by Director General of Pharmaceutical and Food Safety Bureau
  - II. Designation etc. of Orphan Drugs and Orphan Medical Devices
    - Guideline, Detailed description of application, procedure and criteria etc.

Criteria for Orphan Designation in Japan

1) **The number of Patients** (similar to Prevalence in EU) **Patients is less than 50,000 in Japan (less than 3.9/10,000 population)**

2) **High priority in health care needs**

   a) No alternative drugs and/or medical interventions are available (similar to "unmet needs" in EU)

   b) Extremely higher efficacy and/or safety compare to already approved products (similar to "significant benefit" in EU in some extent)

3) **High Possibility of Development**

   Theoretical bases for the application & feasible development plan

Incentives for Orphan Designation

1) Administrative and Scientific Advices
2) Preferential protocol assistance

   & priority review
3) Grant aid for research expenses
4) Authorisation for tax deduction
5) Reduction of application fee
6) Extension of re-examination period

Processes of Orphan Designation -1-

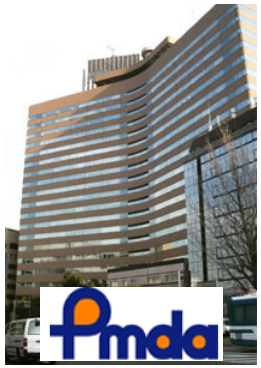
Pref. Governor

General Public

Sponsor

(1) Application of pre-submission meeting

(2) Pre-submission meeting



PAFSC

New Drug1

New Drug 2

Med. Dev & Diagnostics

Processes of Orphan Designation -2-

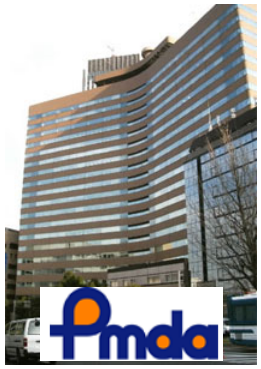
Sponsor

Pref. Governor

General Public

(3)-1 Submission of application

(4)-1 Send the application for review



(4)-3 Review Report

(4)-2 Review & Evaluation

(5)-1 Refer to the PAFSC

(5)-2 Discussion

(5)-3 Recommendation

PAFSC
New Drug1
New Drug 2
Med. Dev & Diagnostics

(3)-2 Validation of Application

Processes of Orphan Designation -3-

EUROPEAN MEDICINES AGENCY

Sponsor

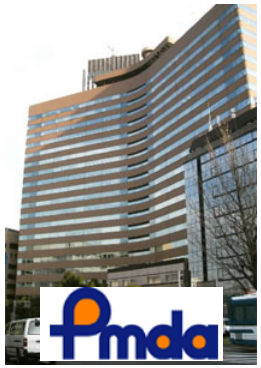
Pref. Governor

General Public

Post designation Incentives requests etc

(6)-2 Notification

(6)-2 Notification (by Gov. Gazette & Document)



(6)-1 Administrative works

PAFSC

New Drug1

New Drug 2

Med. Dev & Diagnostics

Processes of Orphan Designation -Review-

Sponsor

Pref. Governor

General Public

review

Validation

Discuss

MHLW

PAFSC
New Drug1
New Drug 2
Med. Dev & Diagnostics

Processes of Orphan Designation -Review-

EUROPEAN MEDICINES AGENCY

General Public

Sponsor

Public Assessment Report

Rapporteur

Co-rapporteur

The COMP

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Difference between EU & Japan

- No specific Division/Section, Committee in Japan.
- “High possibility of Development” is one of the designation criteria in Japan.
- Designated products are automatically obtained Priority Review in Japan.
- No specific “Time clock” is set in Japan.
- 1 step procedure in Japan whilst 2 step procedure in EU (Re-evaluation of orphan status at the licencing)