Brief Instructions for the Application Form of Orphan Drug Designation

Form 107 (1): Application for Orphan Drug Designation

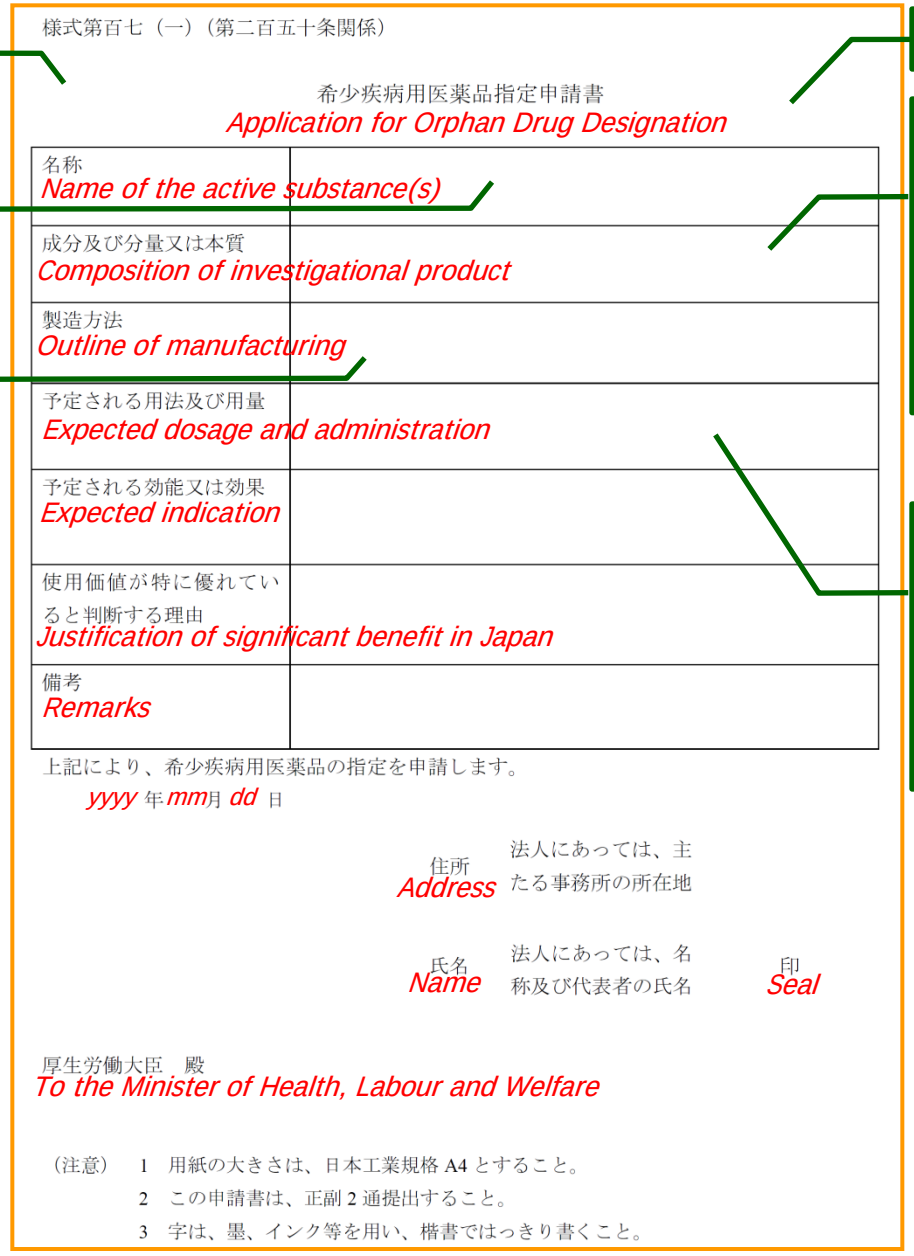
Name of the active substance(s) :
- Fill out using JAN (Japanese Accepted Names for Pharmaceuticals), INN (International Nonproprietary Name) or scientific name, in principle.
- Applicant can search for JAN on following website.
  http://moldb.nihs.go.jp/jan/index.aspx

Outline of manufacturing:
- A brief description of manufacturing should be given.

<Example 1>
YY (active substance) produced by chemical syntheses is prepared to the tablet.

<Example 2>
YY (active substance) is a recombinant human interferon gamma, which is produced in ZZ cells.

<Example 3>
YY (active substance) can be isolated from the human blood.



様式第百七（一）（第二百五十条関係）

希少疾病用医薬品指定申請書
Application for Orphan Drug Designation

名称 *Name of the active substance(s)*	
成分及び分量又は本質 *Composition of investigational product*	
製造方法 *Outline of manufacturing*	
予定される用法及び用量 *Expected dosage and administration*	
予定される効能又は効果 *Expected indication*	
使用価値が特に優れていると判断する理由 *Justification of significant benefit in Japan*	
備考 *Remarks*	

上記により、希少疾病用医薬品の指定を申請します。
yyyy 年 *mm* 月 *dd* 日

住所 *Address*　法人にあっては、主たる事務所の所在地

氏名 *Name*　法人にあっては、名称及び代表者の氏名　印 *Seal*

厚生労働大臣　殿
To the Minister of Health, Labour and Welfare

（注意）　1　用紙の大きさは、日本工業規格 A4 とすること。
2　この申請書は、正副 2 通提出すること。
3　字は、墨、インク等を用い、楷書ではっきり書くこと。

Fill out this form in Japanese.

Composition of investigational product:
- A brief description of the investigational product(s) (formulation, strength) should be given.

<Example>
One tablet contains XX mg of YY (active substance).

Expected dosage and administration:
- Fill out expected dosage and administration.

<Example>
The usual dose is XX mg daily, taken orally in the morning or evening.

Brief Instructions for the Application Form of Orphan Drug Designation

様式第百七（一）（第二百五十条関係）

希少疾病用医薬品指定申請書

Application for Orphan Drug Designation

名称 *Name of the active substance(s)*	
成分及び分量又は本質 *Composition of investigational product*	
製造方法 *Outline of manufacturing*	
予定される用法及び用量 *Expected dosage and administration*	
予定される効能又は効果 *Expected indication*	
使用価値が特に優れていると判断する理由 *Justification of significant benefit in Japan*	
備考 *Remarks*	

上記により、希少疾病用医薬品の指定を申請します。

yyyy 年 *mm* 月 *dd* 日

住所 *Address*　法人にあっては、主たる事務所の所在地

氏名 *Name*　法人にあっては、名称及び代表者の氏名　印 *Seal*

厚生労働大臣　殿

To the Minister of Health, Labour and Welfare

（注意）　1　用紙の大きさは、日本工業規格 A4 とすること。
2　この申請書は、正副 2 通提出すること。
3　字は、墨、インク等を用い、楷書ではっきり書くこと。

Expected indication:

- Fill out expected indication.

<Note>

- In principle, it isn't acceptable if applicant confine the indication with placing a provision on it such as "life-threatening", "serious", "severe" or "other treatments have no effect" and limit the prevalence of the disease under 50,000 so that you can get preferential treatment for Orphan drugs, unless you can provide clear medical/pharmaceutical justification for placing such provision on the indication.

Remarks:

- Fill out name, telephone number, facsimile number and e-mail address of contact person.
- If there is any other special information of this product, applicant should fill in this column.

Submission date for the designation application (year, month, day)

Justification of significant benefit in Japan:

- Description of following points should be given.
  - A) Description of the target disease
    - ✓ Summary of the cause and symptom
    - ✓ Number of patients (prevalence of the condition)
    - ✓ Justification as to why existing methods are not satisfactory
  - B) Medical Plausibility
    - ✓ Mechanism of action
    - ✓ Clinical data
  - C) Summary of current regulatory or development status, and marketing history, out of Japan.
  - D) Summary of current development status and plan of the product in Japan
- If there is not enough space, applicants may explain above things on the separate sheet and attach it.

Permanent address of applicant

Name of the company and name of delegate

<Note>

1. Size of this form shall be A4.
2. Submit an original and two copies of the application for designation.
3. Writing letters should be readable.