

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

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 法人にあつては、主たる事務所の所在地

氏名
Name 法人にあつては、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.

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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)

様式第七 (一) (第二百五十条関係)

希少疾病用医薬品指定申請書

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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.