EUROPEAN COMMISSION
HEALTH AND CONSUMERS DIRECTORATE-GENERAL

Health systems and products
Medicinal products – authorisations, EMA

Annex to Guideline on the format and content of applications for designation as orphan medicinal products and on the transfer of designations from one sponsor to another,date

APPLICATION
for
ORPHAN MEDICINAL PRODUCT
DESIGNATION

DECLARATION and SIGNATURE

Name of the active substance(s):

Sponsor:

Unified Product Identifier number (UPI)

It is hereby confirmed that all data required for the designation of this medicinal product as an orphan medicinal product have been included in the dossier.

It is hereby confirmed that the summaries provided in the application are an accurate account of the data obtained by the sponsor.

_____

(Signature(s) and function of sponsor) _____

(Place and date)

APPLICATION FORM

This application form is to be used to apply for the designation of a medicinal product **for human use** as an orphan medicinal product, according to Regulation (EC) No 141/2000 of 16 December 1999 and Commission Regulation (EC) No 847/2000. The application should be submitted to the European Agency for the Evaluation of Medicinal Products (EMEA).

NOTE: PLEASE CONSULT THE 'GUIDELINE FOR THE FORMAT AND CONTENT OF APPLICATIONS FOR DESIGNATION AS ORPHAN MEDICINAL PRODUCTS (ENTR/6283/00)' WHEN COMPLETING THIS FORM.

I. CRITERIA FOR DESIGNATION

Note: The following sections should be ticked (√) and completed as appropriate.

I.1. THIS APPLICATION CONCERNS:

Note: A sponsor requesting designation of a medicinal product as an orphan medicinal product must request designation before an application for marketing authorisation is made. A request for designation may, however, be made for a new indication for an already authorised medicinal product

☐ **I.1.1. AN ACTIVE SUBSTANCE NOT CURRENTLY AUTHORISED IN THE UNION**

☐ **I.1.2. AN ACTIVE SUBSTANCE CURRENTLY AUTHORISED IN THE UNION**

*Note: The indication for which orphan designation is sought in this application must be **different** to that currently authorised*
If you are the holder of an existing marketing authorisation in the Union for this product, please provide details of the currently authorised indication and the type of marketing authorisation below:

I.1.2.1 Authorised indication(s)

I.1.2.2 Type of marketing authorisation *(tick and complete as appropriate)*

❍ CENTRALISED (according to Regulation (EC) No 726/2004)

Tradename: .....
Date of authorisation: ⌊_⌊_⌋ ⌊_⌊_⌋ ⌊_⌊_⌋⌋
Marketing authorisation number(s): .....
Marketing authorisation holder: .....

❍ MUTUAL RECOGNITION (according to Article 28 of Directive 2001/83/EC)

Reference Member State: .....
Date of authorisation: ⌊_⌊_⌋ ⌊_⌊_⌋ ⌊_⌊_⌋
Marketing authorisation holder: .....
Concerned Member State(s) (specify):

☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐
AT BE BG CY CZ DE DK EE EL ES FI FR HU HR IS IE
☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐
IT LI LT LU LV MT NL NO PL PT RO SE SI SK

Please attach details of tradename(s) and marketing authorisation number(s)

❍ NATIONAL PROCEDURE

Member State(s) where authorised (specify):

☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐
AT BE BG CY CZ DE DK EE EL ES FI FR HU HR IS IE
☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐ ☐
IT LI LT LU LV MT NL NO PL PT RO SE SI SK

Marketing authorisation holder: .....
Please attach details of tradename(s) and marketing authorisation number(s)

☐ **I.1.3. CHANGE OF AN EXISTING DESIGNATION**

Note: A sponsor holding already a designation of a medicinal product as an orphan medicinal product may request to amend its designation for example to change the condition. The number of the designation should be provided.

I.2. THIS APPLICATION IS IN ACCORDANCE WITH THE FOLLOWING PARAGRAPHS IN ARTICLE 3, REGULATION (EC) 141/2000

Note: Both sections I.2.1 and I.2.2 should be completed for all designation applications, by ticking (√) as appropriate.

I.2.1. ARTICLE 3(1)(a), PARAGRAPHS 1 OR 2 (PLEASE TICK EITHER PARAGRAPH 1 OR 2)

❍ PARAGRAPH 1 - PREVALENCE OF A CONDITION IN THE UNION

Note: For the documentation submitted in support of this application (see Table of Contents p.9).
Sections A(1-4); B(1), B(3) should be completed.

❍ PARAGRAPH 2 - POTENTIAL FOR RETURN ON INVESTMENT

Note: For the documentation submitted in support of this application (see Table of Contents p.10).
Sections A(1-4); B(2-3); C(1-5) should be completed.

I.2.2. ARTICLE 3(1)(b), EXISTENCE OF OTHER METHODS OF DIAGNOSIS, PREVENTION OR TREATMENT (PLEASE CHOSE ONE OPTION)

❍ NO OTHER METHODS EXIST IN THE UNION

Note: For the documentation submitted in support of this application (see Table of Contents p.10).
Section D(1) should contain a statement that no other methods currently exist.

❍ OTHER METHODS EXIST BUT ARE NOT CONSIDERED SATISFACTORY

Note: For the documentation submitted in support of this application (see Table of Contents p.10). Sections D(1) and D (2) should be completed.

❍ OTHER SATISFACTORY METHODS EXIST BUT THIS MEDICINAL PRODUCT WILL BE OF SIGNIFICANT BENEFIT TO THOSE AFFECTED BY THE CONDITION

Note: For the documentation submitted in support of this application (see Table of Contents p.10). Section D(1) and D (3) should be completed

II. DESIGNATION APPLICATION PARTICULARS

II.1. Name

II.1.1 Name of the active substance(s):

Note: ***Only one name should be given in the following*** *order of priority: INN[1], Ph.Eur., National Pharmacopoeia, common name, scientific name*
Please indicate in brackets after the name whether the name given is the recommended INN, the PhEur name, or the common name etc.

II.2. Proposed indication and ATC code

II.2.1 Proposed indication:

Note: If more than one indication is applied for, ***separate applications*** *should be submitted* ***for each indication.*** *The dossier should contain a more detailed description of the condition in Section A and a summary of the development of the product in Section E (see Table of Contents for Remainder of Dossier p.9)*

II.2.2 Pharmacotherapeutic group (Please use current ATC code if known)**:**

ATC Code: **Group:**

□ Please indicate when the ATC Code is pending

II.3. Tradename, Strength, pharmaceutical form and route of administration

Note: For products that are in the early stages of development it may not be possible to complete this section.

[1] The INN should be accompanied by its salt or hydrate form if relevant

II.3.1 Proposed Tradename of the medicinal product in the Union:

II.3.2 Strength(s) and Pharmaceutical form(s) (use current list of standard terms - European Pharmacopoeia)

Strength(s) **Ph. Form(s)**

II.3.3 Proposed route(s) of administration (use current list of standard terms - European Pharmacopoeia)

II.4. Sponsor / Contact person

II.4.1 Sponsor:

Name or corporate name of sponsor:
Address:
Country:
Telephone:
E-Mail:
Contact person at sponsor's premises:

Attach proof of establishment of the sponsor in the EEA

II.4.2 For sponsors whose main business is operated from outside the Union, address of those premises and a contact name

Name or corporate name of sponsor:
Contact name:
Address:
Country:
Telephone:
E-Mail:

II.4.3 Person/company authorised for communication on behalf of the sponsor during the procedure:

Name of contact:
Address:
Country:

☐ If different to II.4.1 above,
Append a letter of authorisation

Telephone:
E-Mail:

II.4.4 Person/company for communication between the sponsor and the Agency after designation if different from II.4.1:

Name:
Address:
Country:
Telephone:
E-Mail:

☐ If different to II.4.1 above,
Append a letter of authorisation

II.5 Manufacturers

Note: For products that are in the early stages of development it may not be possible to complete section II.5.2.

II.5.1 Name of Manufacturer(s) and site(s) of manufacture of the finished medicinal product:

Name:
Address:
Country:
Telephone:
E-Mail

III OTHER INFORMATION

III.1 Has scientific advice been given by the CHMP for this medicinal product?

☐ yes ☐ no

If yes,

Date:
Reference of the scientific advice letter:
Append a copy of the scientific advice letter

III.2 Do you intend to seek protocol assistance for this medicinal product?

☐ yes ☐ no

If yes, when?

III.3 Details of planned submission of application for marketing authorisation *(if known)*?

Planned submission date:

Do you intend to request a fee reduction? ☐ yes ☐ no

III.4 Has the sponsor SME status?

☐ yes ☐ no

III.5 1.1. Has the product been subject to a paediatric investigation plan submission?

☐ yes ☐ no

III.6 Has the product been subject to one of the following procedures for advanced therapy medicinal products (ATMP)?

☐ Recommendation on classification yes ☐ Certification of quality and non-clinical data ☐ no

III.7 Do you consider your product as an innovative medicinal product? (please see definition on 'Guideline concerning the optional scope of the centralised procedure in accordance with Article 3(2)(b) of Regulation (EC) No 726/2004')

☐ **No** ☐ **Therapeutic innovation** ☐ **Scientific innovation** ☐ **Technical innovation**

TABLE OF CONTENTS

FOR REMAINDER OF APPLICATION

This table of contents/checklist is to be used as a guide to complete the documentation to be submitted in an application for designation of a medicinal product for human use as an orphan medicinal product, according to Regulation (EC) No 141/2000 of 16 December 1999 and Commission Regulation (EC) No 847/2000.

NOTE: PLEASE CONSULT THE 'GUIDELINE FOR THE FORMAT AND CONTENT OF APPLICATIONS FOR DESIGNATION AS ORPHAN MEDICINAL PRODUCTS (ENTR/6283/00)' WHEN PREPARING THE APPLICATION.

SECTION	***CHECKLIST*** *(tick ☐, as appropriate)*		***INDEX***
A) DESCRIPTION OF THE CONDITION			
1. List of abbreviation	☐ Included		Page___ to____
2. Details of the condition.	☐ Included		Page___ to____
3. Proposed therapeutic indication.	☐ Included		Page___ to____
4. Medical plausibility.	☐ Included		Page___ to____
5. Justification of the life-threatening or debilitating nature of the condition.	☐ Included		Page___ to____

Note: - Section A(1-4) should be completed for all applications.

SECTION	***CHECKLIST*** *(tick ☐, as appropriate)*		***INDEX***
B) PREVALENCE OF THE CONDITION			
1. Prevalence of the orphan disease or condition in the Union.	☐ Included	☐ Not Applicable	Page___ to____
2. Prevalence and incidence of the condition in the Union.	☐ Included	☐ Not Applicable	Page___ to____
3. Information on participation in other EU projects.	☐ Included		Page___ to____

Note: - Section B (1) should be completed for applications submitted in accordance with Article 3(1)(a) paragraph 1
- Section B (2) should be completed for applications submitted in accordance with Article 3(1)(a) paragraph 2
- Section B (3) should be completed for all applications

SECTION	*CHECKLIST* *(tick ☐, as appropriate)*		*INDEX*
C) POTENTIAL FOR RETURN ON INVESTMENT			
1. Grants and tax incentives.	☐ Included	☐ Not Applicable	Page___ to____
2. Past and future development costs.	☐ Included	☐ Not Applicable	Page___ to____
3. Production and marketing costs.	☐ Included	☐ Not Applicable	Page___ to____
4. Expected revenues	☐ Included	☐ Not Applicable	Page___ to____
5. Certification by registered accountant.	☐ Included	☐ Not Applicable	Page___ to____

Note: - This section should only be completed for applications submitted in accordance with Article 3(1)(a) para 2

SECTION	*CHECKLIST* *(tick ☐, as appropriate)*		*INDEX*
D) OTHER METHODS FOR DIAGNOSIS, PREVENTION OR TREATMENT OF THE CONDITION			
1. Details of any existing diagnosis, prevention or treatment methods.	☐ Included		Page___ to____
2. Justification as to why the methods are not considered satisfactory.	☐ Included	☐ Not Applicable	Page___ to____
3. Justification of significant benefit.	☐ Included	☐ Not Applicable	Page___ to____

Note: - Section D (1) should be completed for all applications
- Section D (2) or D (3) should be completed as appropriate.

SECTION	*CHECKLIST* *(tick ☐, as appropriate)*		*INDEX*
E) DESCRIPTION OF THE STAGE OF DEVELOPMENT			
1. Summary of the development of the product.	☐ Included		Page___ to____
2. Details of regulatory status and marketing history in non EU countries.	☐ Included		Page___ to____

Note: - This section should be completed for all applications.

SECTION	*CHECKLIST* *(tick ☐, as appropriate)*		*INDEX*
F) BIBLIOGRAPHY			
This section should contain all published references referred to in the sections A to D above.	☐ Included		Page___ to____