Sections A to E (scientific part)

The purpose of this template is to facilitate sponsors in completing the scientific part (sections A-E) of the application for orphan designation.

| **Related documents:** |  |
| --- | --- |
| For general guidance on the content of the application sponsors should refer to the [*Guideline on the format and content of applications for designation as orphan medicinal products and on the transfer of designations form one sponsor to another 2022/C 440/02*](https://eur-lex.europa.eu/legal-content/EN/TXT/?uri=uriserv:OJ.C_.2022.440.01.0002.01.ENG)*.* The guideline explains in detail what each section of the scientific part of the application should incorporate. | |
| **In addition the sponsor is advised to refer to guidance available at the EMA website:**  [**Orphans: Regulatory and procedural guidance and forms**](https://www.ema.europa.eu/en/human-regulatory/research-development/orphan-designation/applying-designation/orphans-regulatory-procedural-guidance-forms) | |

Please fill in the template without amending the styles and the format. Please do not use hyperlinks. Please see the Annex for the formats to be used when inserting tables, figures, etc.

<Date>

| Submission ID | EMA/OD/<Text> |
| --- | --- |
| Active substance[s]: | <Text> *as per submission via the online platform*  *Please refer to* [*List of substances*](https://iris.ema.europa.eu/substances/) |
| Orphan condition | <Text> *as per submission via the online platform* |
| Translations required with the submission of an application | [Link to template](https://view.officeapps.live.com/op/view.aspx?src=https%3A%2F%2Fwww.ema.europa.eu%2Fen%2Fdocuments%2Ftemplate-form%2Ftemplate-translations-required-submission-application-orphan-medicinal-product-designation_en.doc&wdOrigin=BROWSELINK) |

Table of contents

[List of abbreviations 3](#_Toc107924310)

[Sections A-E 4](#_Toc107924311)

[A. Description of the condition 4](#_Toc107924312)

[A1. Details of the condition 4](#_Toc107924313)

[A2. Proposed orphan indication 4](#_Toc107924314)

[A3. Medical plausibility 4](#_Toc107924315)

[A4. Justification of the life-threatening or debilitating nature of the condition 4](#_Toc107924316)

[B. Prevalence of the condition 4](#_Toc107924317)

[B1. Prevalence of the orphan disease or condition in the European Union 4](#_Toc107924318)

[B2. Prevalence and incidence of the condition in the European Union 5](#_Toc107924319)

[C. Potential for return on investment 5](#_Toc107924320)

[C1. Grants and tax incentives 5](#_Toc107924321)

[C2. Past and future costs 5](#_Toc107924322)

[C3. Production and marketing costs 5](#_Toc107924323)

[C4. Expected revenues 5](#_Toc107924324)

[C5. Certification by registered accountant 5](#_Toc107924325)

[D. Other methods for diagnosis, prevention or treatment of the condition 5](#_Toc107924326)

[D1. Details of any existing diagnosis, prevention or treatment methods 5](#_Toc107924327)

[D2. Justification as to why methods are not satisfactory 5](#_Toc107924328)

[D3. Justification of significant benefit 5](#_Toc107924329)

[E. Description of the stage of development 5](#_Toc107924330)

[E1. Summary of the development of the product 5](#_Toc107924331)

[E2. Details of current regulatory status and marketing history in the EU and non EU countries 6](#_Toc107924332)

[F. Bibliography 7](#_Toc107924333)

List of abbreviations

An abbreviations list must be provided with each application.

<Text>

Sections A-E

A. Description of the condition

A1. Details of the condition

<Text>

* Definition

<Text>

* Aetiology

<Text>

* Specific characteristics; pathophysiological, histopathological, clinical characteristics

<Text>

* Classification

<Text>

* Diagnosis and symptoms

<Text>

A2. Proposed orphan indication

<Text>

A3. Medical plausibility

A.3.1. Active substance: description of the medicinal product, pharmacological class and mode of action

<Text>

A.3.2. Plausibility of the orphan condition; data with the specific product as applied for designation in specific models or in patients affected the condition

<Text>

A4. Justification of the life-threatening or debilitating nature of the condition

<Text>

B. Prevalence of the condition

B1. Prevalence of the orphan disease or condition in the European Union

<Text>

B2. Prevalence and incidence of the condition in the European Union

<Text>

C. Potential for return on investment

<Text> or Not applicable. (delete C1-C5 if not applicable)

C1. Grants and tax incentives

C2. Past and future costs

C3. Production and marketing costs

C4. Expected revenues

C5. Certification by registered accountant

D. Other methods for diagnosis, prevention or treatment of the condition

D1. Details of any existing diagnosis, prevention or treatment methods

<Text>

D2. Justification as to why methods are not satisfactory

<Text> or Not applicable. (delete as appropriate)

Note that sections D2 and D3 are mutually exclusive.

D3. Justification of significant benefit

<Text> or Not applicable. (delete as appropriate)

E. Description of the stage of development

E1. Summary of the development of the product

<Text>

Quality aspects

Non-clinical aspects

Proof-of concept in relevant model

Pharmacology

Pharmacokinetics

Toxicology

Clinical aspects

Pharmacokinetics

Pharmacodynamics

Clinical efficacy

Dose-response studies and main clinical studies

Clinical studies in applied condition

Planned clinical studies

Clinical safety

Adverse events

Serious adverse events and deaths

E2. Details of current regulatory status and marketing history in the EU and non EU countries

Sponsor’s position:

* An application for marketing authorisation has previously been submitted in the EU[[1]](#footnote-2) for this medicinal product, with the proposed invented name of <(tradename)>,in <indication>. The application was withdrawn in *<*year>.
* This medicinal product product is currently authorised in the EU1, with the invented name of <(tradename)>, for the following indication(s): <indication>
* This medicinal product was not authorised in any country inside or outside the EU at the time of submission of the application.
* Scientific advice or protocol assistance on this medicinal product was given by the CHMP in *<*month/year>.
* In the US, orphan drug status was granted on <date> for <indication>.

Please delete any paragraph above that does not apply.

F. Bibliography

This section should contain all published references referred to in section A to D above and should be submitted together with the application but as a separate file (a single PDF or ZIP file). Where information is printed out from a web-site the date that the web-site has been accessed should be noted.

The preferred format for cross-referencing published literature in Section A-E of the application is by the lead author and year e.g. (Smith et al, 2002). Please do not use hyperlinks.

<Text>

Annex (to be deleted before submitting section A to E.)

Please click on styles and formatting icon and use the relevant formats for tables, figures, bullets, number lists and footnotes (to be inserted, if needed in relevant sections and not as an annex).

1. Table heading

|  |  |  |  |
| --- | --- | --- | --- |
| Table heading rows |  |  |  |
| Table text rows |  |  |  |
|  |  |  |  |

Table note

1. Figure heading

Figure

Figure note

* bullet
* bullet
* bullet
* bullet

1. Number list
2. Number list
3. Number list
4. Number list

Footnote[[2]](#footnote-3) (please see the footnote style below)

1. By the Sponsor or another individual or organisation with whom the Sponsor had or has an agreement regarding the development of the product [↑](#footnote-ref-2)
2. Footnote text [↑](#footnote-ref-3)