Medicines & Healthcare products Regulatory Agency

MHRA

United Kingdom Orphan Drug Designation Application Form

Sections A to E (scientific part)

<Date>

Submission ID	UK/OD/<Text>
Active substance[s]:	<Text>
Orphan indication	<Text>

Table of contents

Medicines & Healthcare products Regulatory Agency

MHRA

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 Great Britain

<Text>

B2. Prevalence and incidence of the condition in the Great Britain

<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 UK and non-UK countries

Applicant's position:

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).

Table 1. Table heading

Table heading rows			
Table text rows			

Table note

Figure 1. Figure heading

Figure

Figure note

- bullet
- bullet
- bullet
- bullet

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