9 October 2023

EMA/76018/2021

European Medicines Agency

ITF Briefing Meeting

Briefing Document

This briefing document needs to be completed for your:

- Product / Substance (section 1)

**AND/OR**

- Method / Methodology / Technology (section 2)

Summary: max. 3 pages

Total briefing document: max. 30 pages (excluding annexes)

# Data protection notice

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|  |  |
| --- | --- |
| **Applicant:** |  |
| **Document version:** |  |
| **Date:** |  |

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[**Bracketing convention:**

[Text] Explanation and guidance, to be deleted before sending the final version of the briefing document to EMA.

{text} Information that is required to be filled in.

<text> Text to be selected or deleted as appropriate.

**Headings**:

Standard headings in the template should be used whenever possible; if it is considered necessary to deviate from the pre-specified headings to accommodate product/method/technology-specific requirements, alternative or additional headings/sections may be considered.

**Formatting convention:**

Verdana 9 pt., single space, justified.

**References convention:**

Footnotes are preferred for citation of literature references. Alternatively, the format (first author <et al.>, title, publication year) is recommended.]

List of figures

List of tables

List of abbreviations

[Any acronyms and abbreviations used should also be defined the first time they appear in the text]

1. Product / Substance

|  |  |
| --- | --- |
| Invented Name: |  |
| Active substance: |  |
| Pharmaco-therapeutic group: |  |
| Intended indication(s): |  |

* 1. Summary

*[Address all relevant/applicable elements outlined below, regardless of the topics for discussion (Upper limit for your summary: 3 pages)]*

Background information on the condition to be treated

[Outline main features of the condition and current standard therapy, referring to relevant publications]

Background information on the product

[Include mode of action, chemical structure and pharmacological classification as applicable.

Please specify the proposed wording for the intended indication/use, posology, and any special precautions or recommendations for use of the product (including a possible risk management strategy)]

<Quality development>

[Relevance and level of detail included may vary depending on the scope of the request. Include special pharmaceutical aspects, if any, e.g. novel delivery system, etc.]

<Non-clinical development>

[Relevance and level of detail included may vary depending on the scope of the request. Proof-of-concept and main toxicological findings would be informative]

*<*Clinical development>

[Introduce and describe the status of the clinical development programme. A tabulated summary of completed, ongoing and planned clinical trials would be informative.

Briefly highlight if:

* scientific advice has been previously requested from the CHMP, national or non-EU regulators (e.g. FDA)
* relevant CHMP guidance/advice has been followed or if any deviations have been made or proposed.
* there is a Paediatric Investigation Plan (with or without deferral or waiver).
* there is availability or need for development in other special populations such as the elderly, male/female and ethnic minorities]

Regulatory status

[Describe the worldwide regulatory status of the product/substance. Add any existing Marketing Authorisation, variation, or planned Marketing Authorisation Application timelines, indicating planned type (e.g. full/mixed dossier; advanced therapy, biosimilar, generic/hybrid/ product)]

Rationale for seeking advice

[Describe the scope of the topics for discussion and the rationale for the advice request, e.g. clinical/non-clinical/quality/significant benefit/similarity/conditional approval/exceptional circumstances]

* 1. Topics for discussion (maximum 8)

[Topics for discussion should be phrased unambiguously. The scope should be carefully considered in order to ensure an open discussion.

The wording of the topics should avoid extended reference to the justifications, which should be discussed in the Applicant position. Topics should ideally start or end with e.g. “What are the experts’ opinions / suggestions on…?”).

Topics for discussion should be numbered sequentially.

**IMPORTANT:**

Each topic for discussion should be followed by a corresponding, separate Applicant’s position, including a justification of the chosen approach.

All key information about the topic should be sufficiently discussed, so the Applicant’s position can function as a ‘stand-alone’ argument.

Issues to be covered could include the following: context and proposal, other options (potentially) considered together with a critical discussion on the relative merits and drawbacks of various approaches, possible consequences and eventual measures to ameliorate these.

**<A. *Chemical, Pharmaceutical and Biological* development>**

**Question 1**

{text}

**Applicant’s position**

{text}

**Question 2**

{text}

**Applicant’s position**

{text}

**<B. Toxico-Pharmacological development>**

**Question {X}**

{text}

**Applicant’s position**

{text}

**<C. Clinical development>**

**Question {X}**

{text}

**Applicant’s position**

{text}

**<A/B. Multidisciplinary: Chemical, Pharmaceutical, Biological and Toxico-Pharmacological development>**

{text}

**Applicant’s position**

{text}

**<B/C. Multidisciplinary: Toxico-Pharmacological and Clinical development>**

{text}

**Applicant’s position**

{text}

* 1. Background information

[Give a comprehensive scientific overview of the product/substance, providing detailed relevant systematic information.

All key information about the topic should also be included here as well as in the Applicant’s position, which should function as a ‘stand-alone’ argument

The proposed list of subsections below is neither exhaustive nor mandatory. The relevance or applicability of each subsection may vary.

Additional details can be included in study protocols, study reports, investigators’ brochure provided as annexes. The use of tabulated overviews and graphs is encouraged.]

Quality background information

<Active substance>

<Finished product>

Non-clinical background information

[We recommend including a tabulated overview of all non-clinical studies (completed, ongoing and planned), including study number, main design features and GLP status. Main findings and safety margins may be described in the narrative.]

<Pharmacology>

<Pharmacokinetics>

<Pharmacodynamics>

<Toxicology>

Clinical background information

[A tabular overview of all clinical studies (completed, ongoing and planned), including study number, main design features, patient number and characteristics, etc., should be included here, if available and not provided elsewhere. Whilst the focus should be kept on the intended indication, the development in other indications could be briefly summarised, where relevant.]

<Clinical pharmacology>

<Pharmacokinetics>

<Pharmacodynamics>

<Clinical efficacy>

[A general overview of the clinical development program should be based on a comprehensive discussion of e.g. the main clinical results so far, dose-response, exploratory trials, special populations, supportive and pivotal clinical studies, and any analyses performed across trials (pooled and meta-analysis). The discussion should identify the most important findings and challenges in the clinical development program, and its compliance with legal requirements, relevant clinical guidelines, previous scientific advice (sufficiently justifying any deviations), etc. Information on the geographical distribution of centres participating in the pivotal clinical studies can be reflected in this section.]

<Clinical safety>

[A general overview of the safety profile of the product should be based on a comprehensive discussion of e.g. patient exposure (safety database), adverse events observed so far, serious adverse events and deaths, laboratory findings, safety-related discontinuations, specific safety findings, immunological events, safety in special populations, etc.]

* 1. Supplementary information

List of references

[Any potentially relevant publications included in the list of references should be annexed (in PDF format, either collated as a single document or - if provided as single files - clearly identified and compiled compressed files, for convenience).]

List of annexes

[Annexes should include any information potentially relevant to the questions, e.g.:

* Investigators’ brochure
* Study protocols (final, draft or outline/synopsis)
* Study reports (final/draft/synopses)
* Previous scientific advice received (e.g. CHMP Scientific advice/Protocol Assistance, any relevant official correspondence and meeting minutes with National Competent Authorities in EU-Member States, FDA and other non-EU Authorities)
* Relevant guidelines (non-EMA)
* Documents related to Orphan Drug Designation (e.g. COMP summary report)
* Documents relating to Marketing Authorisation Application e.g. Day 120 List of Questions, Letter of undertaking
* Documents related to Paediatric Investigation Plans (e.g. PDCO summary report, opinion)
* Contract/agreement consultant/CRO - sponsor
* Literature references]

1. Method / Methodology / Technology
   1. Summary

[Address all relevant/applicable elements outlined below, regardless of the topics for discussion (Upper limit for your summary: 3 pages)]

<Background information on the condition to be treated>

[Outline main features of the condition and current standard therapy, referring to relevant publications]

Background information on the method / methodology / technology

[Please specify the proposed wording for the intended indication/use, and any special precautions or recommendations for use of the method / methodology / technology (including a possible risk management strategy).]

Regulatory status

[Describe the worldwide regulatory status of the method / methodology / technology.]

Rationale for seeking advice

[Describe the scope of the topics for discussion and the rationale for the advice request.]

* 1. Topics for discussion (maximum 8)

[Topics for discussion should be phrased unambiguously. The scope should be carefully considered in order to ensure an open discussion.

The wording of the topics should avoid extended reference to the justifications, which should be discussed in the Applicant’s position.

Topics should ideally start or end with e.g. “What are the experts’ opinions / suggestions on…?”).

Topics for discussion should be numbered sequentially.

**IMPORTANT:**

Each topic for discussion should be followed by a corresponding, separate Applicant’s position, including a justification of the chosen approach.

All key information about the topic should be sufficiently discussed, so the Applicant’s position can function as a ‘stand-alone’ argument.

Issues to be covered could include the following: context and proposal, other options (potentially) considered together with a critical discussion on the relative merits and drawbacks of various approaches, possible consequences and eventual measures to ameliorate these.

**Question X**

{text}

**Applicant’s position**

{text}

**Question X**

{text}

**Applicant’s position**

{text}

* 1. Background information

[Give a comprehensive scientific overview of the method / methodology / technology, providing detailed relevant systematic information.

All key information about the topic should also be included here as well as in the Applicant’s position, which should function as a ‘stand-alone’ argument

The proposed list of subsections below is neither exhaustive nor mandatory. The relevance or applicability of each subsection may vary.

Additional details can be included in study protocols, study reports, investigators’ brochure provided as annexes. The use of tabulated overviews and graphs is encouraged.]

Characteristics of the proposed novel method / methodology / technology

[Elaborate on the scientific rationale for the proposed novel method / methodology / technology, i.e. biological, pharmacological, (patho)physiological or technological background.]

Context of Use

[The disease/condition/experimental setting that is associated with the novel method / methodology / technology.

Describe the intended use of the novel method / methodology / technology in medicinal development and use, and how the novel method / methodology / technology is to be integrated in drug development and regulatory review.

Summarize the signs and symptoms, pathophysiology, risk factors and epidemiology, diagnosis, established therapy, and prognosis of the condition, if applicable. Focus on factors that contribute to improved medicinal development or treatment outcome e.g. early diagnosis, risk prediction, detection of drug related adverse effects, determination of therapeutic response and optimization of therapy.]

The need and impact of proposed novel method / methodology / technology

[Describe the potential impact of the proposed novel method / methodology / technology on current regulatory guidelines, if applicable.

Describe the limitations to the proposed novel method / methodology / technology.]

Sources of data and major findings

[This section is intended to provide a detailed overview and critical analysis/interpretation of the novel method / methodology / technology development programme (including relevant experimental data if applicable). Consider including evidence from published literature, if applicable.]

Remaining gaps and a brief overview of how these will be addressed (if applicable)

[Describe the remaining gaps and how these will be addressed. Include detailed protocol(s) of planned studies in the appendices (if applicable).]

Currently available similar method / methodology / technology

[Describe the currently available method / methodology / technology and the utility and limitations of currently available methods/parameters that are used for the intended application(s) of the proposed novel method / methodology / technology and the added benefit of the proposed novel method / methodology / technology.]

<Technology readiness level>

[Describe the current stage of development of the technology. Where appropriate, mention key milestones that led to the current stage (e.g. prototype, field trials, pilot studies, etc.)

<https://ec.europa.eu/research/participants/data/ref/h2020/wp/2014_2015/annexes/h2020-wp1415-annex-g-trl_en.pdf>]

Description of Action (DoA)

[For consortia applying to grants, this is the technical document including the workplan of a project. i.e. in the context of European Commission grants, the Description of Action corresponds to Annex I of the Grant Agreement. Please include this document as an annex if not already provided.]

* 1. Supplementary information

List of references

[Any potentially relevant publications included in the list of references should be annexed (in PDF format, either collated as a single document or - if provided as single files - clearly identified and compiled compressed files, for convenience).]

List of annexes

[Annexes should include any information potentially relevant to the questions, e.g.:

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* Documents related to Paediatric Investigation Plans (e.g. PDCO summary report, opinion)
* Contract/agreement consultant/CRO - sponsor
* Literature references]