

EUnetHTA Joint Action 3 WP4

**Relative effectiveness assessment of pharmaceutical technologies**

**Scoping Document EUnetHTA REA**

**PTJAXX – [compound] for treatment of [disease]**

Template version 2, April 2020

**Version [x], [day] [month] [year]**

**Disclaimer:** EUnetHTA is supported by a grant from the European Commission. The sole responsibility for the content of this document lies with the authors [submitting manufacturer] and neither the European Commission nor EUnetHTA are responsible for any use that may be made of the information contained therein.

|  |
| --- |
| *NOTE TO AUTHORS OF THIS DOCUMENT*  *Text in grey boxes provide guidance for populating the Letter of Intent template. The boxes should be removed before submitting the Scoping Document to EUnetHTA. In addition, if predefined text requires an update this is marked in yellow.*    *Please follow the layout settings for text, tables and headers as much as possible:*   * Headers Arial 11, bold and italic * Standard text: Arial 10 * Table text: Arial 9 |

**TABLE OF CONTENT**

[1 Background 5](#_Toc37257569)

[1.1 Description of the disease 5](#_Toc37257570)

[1.2 Specification of the population covered by the indication 5](#_Toc37257571)

[2 [Compound – INN & Brand name] 5](#_Toc37257572)

[2.1 Description of the medicine and mechanism of action 5](#_Toc37257573)

[2.2 Method of administration and dosage 5](#_Toc37257574)

[2.3 Marketing authorisation status 5](#_Toc37257575)

[2.4 EU indication 5](#_Toc37257576)

[2.5 Companion diagnostic 5](#_Toc37257577)

[2.6 EU clinical practice and comparators 6](#_Toc37257578)

[2.7 Estimated population size 6](#_Toc37257579)

[2.8 Suggested PICO for the joint assessment 6](#_Toc37257580)

[2.9 Summary table of the studies considering the compound for assessment 7](#_Toc37257581)

[3 Suggested methods for the joint assessment 8](#_Toc37257582)

[4 References 9](#_Toc37257583)

[5 Appendix – Letter of Intent 10](#_Toc37257584)

**LIST OF ABBREVIATIONS**

|  |  |
| --- | --- |
|  |  |
|  |  |
|  |  |
|  |  |
| LoI | Letter of Intent |
| PTJA | Pharmaceutical Joint Assessment |
| (p)MAH | (prospective) Marketing Authorisation Holder |
|  |  |
|  |  |
|  |  |

**PTJAXX – SCOPING DOCUMENT FOR [COMPOUND] FOR [INDICATION]**

|  |
| --- |
| *The scoping document should be used to explain the (p)MAH’s perspective on the PICO (& justification of this), the methods/analysis used and the reasons for their use. When indirect comparisons are part of the (p)MAH’s analysis, the procedure and considerations of such analyses need to be explained in the scoping document. The main goal of the scoping document is to better inform the authors for the scoping F2F meeting and thus should not be a draft Submission Dossier. The scoping document should also not address any results, nor will the authoring team provide any assessment of appropriateness of the suggested methods.*  *Please limit the scoping document to* ***a maximum of 10 pages****. We advise the (p)MAH to add the LoI as an appendix to the scoping document so that you can refer to some of the information already discussed in the LoI.*  *Should the information in the LoI be outdated, we kindly ask the (p)MAH to update the information in the LoI that will be attached to this Scoping Document. Please also highlight in the Scoping Document in case any information in the LoI (and which section) has been updated.* |

# Background

## Description of the disease

|  |
| --- |
| *Please provide us with a brief description of the disease/condition for which the medicine is proposed and anticipated to be indicated.* |

## Specification of the population covered by the indication

|  |
| --- |
| *Please provide us with a brief description of the population that falls under the submitted and anticipated indication.* |

# [Compound – INN & Brand name]

## Description of the medicine and mechanism of action

## Method of administration and dosage

## Marketing authorisation status

|  |
| --- |
| *Please refer to the appendix – Letter of Intent – section 5. Please make sure the information in section 5 of the Letter of Intent is update in case of any changes in the regulatory information and timelines.* |

## EU indication

|  |
| --- |
| *Please state the proposed (submitted to the EMA) and the anticipated indication(s) in the EU. Please do note that the coordination team should be immediately notified if any change is foreseen.* |

## Companion diagnostic

|  |
| --- |
| *Please refer to the Letter of Intent in the appendix. If needed, use this section to provide further information on e.g. the description of the companion diagnostic.* |

## EU clinical practice and comparators

|  |
| --- |
| *Please provide us with a brief summary of the current clinical pathway of the disease/condition for which the medicine is proposed and anticipated to be indicated, highlighting the anticipated comparators in the EU.* |

## Estimated population size

|  |
| --- |
| *Please provide us with the estimated European population size.* |

## Suggested PICO for the joint assessment

|  |
| --- |
| *Please provide us your PICO suggestion(s) of for the joint assessment. If you consider more than one PICO relevant (e. g. different subpopulations require different comparators) please state them by adding additional tables (e.g. PICO 1, PICO 2…).*  *The PICO(s) provided below will be used as a starting point for the PICO the authoring team will develop for the EUnetHTA WP4 PICO Survey. Please note that the EUnetHTA authoring team is free to deviate from the PICO suggested by the (p)MAH.* |

|  |  |  |
| --- | --- | --- |
|  | **PICO 1** | **PICO 2** |
| Population |  |  |
| Intervention |  |  |
| Comparator |  |  |
| Outcomes |  |  |

## Summary table of the studies considering the compound for assessment

|  |
| --- |
| *Please provide a list of all the studies with the compound described in section 2.1 in the EU indication(s) described in section 2.4. Please use the table below and add lines as required.* |

| **Study** | **Included in the regulatory submission dossier** (yes / no) | **Sponsored study**  (yes / no) | **Status** (completed / discontinued / ongoing) | **Study duration** | **Treatment arms** | **Available documents** |
| --- | --- | --- | --- | --- | --- | --- |
|  |  |  |  |  |  | **CSR:** (yes / no)  **Study register entry:** (yes <include link> / no)  **Study results register entry:** (yes <include link> / no)  **Journal publication:** (yes <include link and bibliographic reference> / no)  **Abstract publication:** (yes <include link and bibliographic reference> / no) |
|  |  |  |  |  |  | **CSR:** (yes / no)  **Study register entry:** (yes <include link> / no)  **Study results register entry:** (yes <include link> / no)  **Journal publication:** (yes <include link and bibliographic reference> / no)  **Abstract publication:** (yes <include link and bibliographic reference> / no) |
|  |  |  |  |  |  | **CSR:** (yes / no)  **Study register entry:** (yes <include link> / no)  **Study results register entry:** (yes <include link> / no)  **Journal publication:** (yes <include link and bibliographic reference> / no)  **Abstract publication:** (yes <include link and bibliographic reference> / no) |
| *Etc*. |  |  |  |  |  |  |

# Anticipated methods for data in submission dossier

|  |
| --- |
| *Please only describe your anticipated approach/methods, but do not include any results. This section merely serves for the team to get a better understanding of how the data in the submission dossier will be derived, the authoring team will not give an early assessment on the appropriateness of the suggested methods. Consider explaining:*   * Systematic Literature Review * Feasibility Assessment * Baseline pooling (if relevant) * NMA (if relevant) |

# References

|  |
| --- |
| *Please attach the full-text versions of relevant articles if possible to the scoping document.* |

# Appendix – Letter of Intent

|  |
| --- |
| *Please enclose here the Letter of Intent.*  *Please make sure section 5 – marketing authorization status – is updated accordingly in case the timelines changed* |