



Repurposing pilot project for authorised medicines

Submission Form

(To be submitted to: see Annex to the Q&A on repurposing pilot project – of note for submission to EMA: please use [Eudralink](#))

Active substance(s)	
<i>Provide the name(s) of the active substance(s) that are the subject of repurposing</i>	
Champion	
Champion ¹	<i>Provide the name of the Champion</i>
Contact details	<i>Provide contact details of the contact person (e-mail address, phone number,...)</i>
New therapeutic use targeted	
New proposed condition or indication	<i>Describe</i>
Is the proposed new condition/indication for the authorised active substance distinct to the currently authorised indication(s) listed in section 4.1 of authorised medicinal product(s) in the EEA? ²	<i>Yes/No</i>
Do you hold an orphan designation for the proposed repurposing project?	<i>Yes/No</i>
Authorised medicinal product(s) in the EU/EEA	
Authorised indication(s) (section 4.1 SmPC)	<i>To be listed/summarised</i>
Authorised pharmaceutical form(s)	<i>Section 3 SmPC or an extract of the Public Data from Article 57 database³ can be provided as an annex</i>
Authorisation details (date of first authorisation, MAH name)	<i>Section 9 SmPC or an extract of the Public Data from Article 57 database³ can be provided as an annex</i>

¹ If Champions are established outside the European Economic Area (EEA), it is advisable for Champions developing the products to nominate a contact point within the EEA to facilitate communication between the Authorities and such Champions. This contact point may be the same as the Champion, or not. For collaborative group, one contact point should be established to act as champion on behalf of the association/network.

² In case of product combinations, it is necessary to specify the indications of both.

³ The Article 57 database can be accessed in the following link: <https://www.ema.europa.eu/en/human-regulatory/post-authorisation/data-medicines-iso-idmp-standards/public-data-article-57-database>. It contains all medicines authorised in the European Economic Area (EEA). The Marketing authorisation holders must submit and maintain this information in accordance with European Union (EU) legislation. It can be downloaded as an Excel sheet and filtered by active substance. For further details requested in this section, refer to the following links for centrally authorised medicines (search [Medicines](#)) or nationally authorised medicines (visit the websites of the [national competent authorities](#)).

Has the innovator / brand leader authorised medicinal product been granted a marketing authorisation more than 8 years ago?	<i>Yes/No/Unknown [If possible, provide, , information e.g. product name / MAH(s), date of authorisation and indicate if authorised by a Member State or the European Commission]</i>
Is an authorised medicinal product(s) containing the concerned active substance out of basic patent / supplementary protection certificate (SPC) protection, and data and market exclusivity periods?	<i>Yes/No/Unknown</i>

Champion characteristics	
Please indicate if you are a Not-for-profit organisation as per the definition in footnote ⁴	<i>Yes/No</i>
Please indicate if you are a patient organisation as per the definition in footnote ⁵ :	<i>Yes/No</i>
Please indicate if you are a collaborative group ⁶ (if yes, please provide the composition of the group)	<i>Yes/No</i>
Please indicate if you are an Academic Institution as per the definition in footnote ⁷	<i>Yes/No</i>
<i>For academia only</i> [#]	<i>Yes/No</i>
Is the entity's seat located in the EU, Iceland, Liechtenstein or Norway?	<i>Yes/No</i>
Are you meeting criteria c) of the Annex 'Academia status'	<i>Yes/No</i>

[#] Of note: this information is in light of [fees applicable to academia for an orphan development](#) but is without prejudice of taking part to the repurposing pilot

Applicant's planned approach for scientific advice	
<i>Note: only one pathway should be followed, either EMA or NCA</i>	
National Competent Authority (NCA)	<i>Yes/No</i>
Name of NCA	<i>Please provide the name of the NCA</i>
EMA	<i>Yes/No</i>

⁴ 'Non-profit organisation' or 'non-profit legal entity' should be understood as a legal entity which by its legal form is non-profit-making or which has a legal or statutory obligation not to distribute profits to its shareholders or individual members; 'Legal entity' should be understood as any natural person, or any legal person created and recognised as such under national law, Union law or international law, which has legal personality and which may, acting in its own name, exercise rights and be subject to obligations;

⁵ 'Patient organisations' should be understood as not-for profit organisations which are patient focused, and in which patients or carers (the latter when patients are unable to represent themselves) represent a majority of members in their governing bodies.

⁶ Collaborative groups and European Reference Networks (ERNs) should be understood as virtual networks or associations of persons without legal personality involving healthcare providers and researchers across Europe.

⁷ 'Academia' or 'Academic sector' should be understood as consisting of public or private higher education establishments awarding academic degrees, public or private non-profit research organisations whose primary mission is to pursue research, and international European interest organisations; 'International European interest organisation' should be understood as an international organisation, the majority of whose members are Member States or associated countries, and whose principal objective is to promote scientific and technological cooperation in Europe.

The following elements are important to evaluate the plausibility and feasibility of an authorised medicine repurposing approach. Boxes contain minimum pieces of information that is needed to assess the proposal but descriptions may not be restricted to those elements. It is highly recommended to provide detailed relevant information, including references and annexes (max. limit of 20 pages):

1. Product description & mechanism(s) of action

<Substance type (chemical, biological)/structure, authorised dose and route of administration, reference to official product information of authorised medicines⁸ (if available)>

2. Proposed new condition(s)/indication(s)

Please consider as much as possible some or all of the components in the following mock-up which may be relevant to cover in the targeted indication:

<Diagnostic use >or <Preventive> or <Symptomatic, curative or disease modifying (if applicable)> <treatment of> <{severity criteria if applicable}> <{target disease or condition}> in <{age group}> patients < {restrictions to patient population, if applicable}> <{restrictions in terms of therapeutic option or prior therapy, or other restrictions, if applicable}> <in combination with other medicinal products <{list relevant combinations, if applicable}><in monotherapy>

3. Background information on the disease/population targeted and the unmet medical need

- a) Background of the disease (including seriousness, population, prevalence, etc)
- b) Discussion on the current available treatment(s), with their limitations and disadvantages (reference to clinical guidelines could be useful)
- c) Description of the existing unmet need to be tackled.

4. Claim of major public health interest

Please, provide information on how the new indication may add value from the public health interest point of view

5. Does the product hold sufficient promise to address the unmet medical need described in section 3)?

Please, consider all the following and provide justification for missing information. Different points may have more relevance depending on the specific case (for example, in some cases extensive use in a given indication with proof of efficacy makes less important the completeness of non-clinical data; conversely, completely new uses may discuss more thoroughly plausibility and non-clinical data). Add as much information as possible when available.

- a) Plausibility of mechanism of action / proof-of concept data to support the new condition/indication (e.g. chronic vs. short-term use, different posology, different mode of action, different targeted population)
- b) Preliminary pre-clinical/clinical data / strength of current evidence to address the unmet medical need
- c) Real world data available (post-authorisation studies, registry data, named patient basis, magistral preparation, off-label use)
- d) Indication is included in clinical guidelines or other recommendations such as health technology assessment (HTA)

⁸ See available information through the article 57 database. Follow the instructions in page 1.

- e) Safety profile
- f) Evidence supporting safety in the proposed new condition/indication

6. Please provide tabular overviews of the completed/on-going/planned pre-clinical studies (e.g. study type/objectives, species/strain, mode of administration, doses, number of assays/animals, study duration, outcome variables, GLP conditions)

7. Please provide tabular overviews of the completed/on-going/planned clinical studies (e.g. study type/objectives, species/strain, mode of administration, doses, number of patients, study duration, outcome variables, GCP conditions)

8. Does the Champion consider that further pre-clinical & clinical studies are necessary to demonstrate efficacy and safety in this new indication? If yes, please specify.

Please, discuss here any gap in the pre-clinical and clinical development that should be covered by further research

Pre-clinical

Clinical

9. Does the Champion have resources to conduct additional pre-clinical & clinical studies?

Please, discuss here studies already ongoing or planned to fill the gaps

10. Regulatory status

- a) Previous and planned interactions with regulators
- b) Orphan drug designation: Yes/No/planned/non-applicable
<Delete as appropriate. If yes, give details.>
- c) Potential for Paediatric-use marketing authorisation (PUMA)
- d) Potential for 1-year data exclusivity for a new indication (Article 10(5) of Directive 2001/83/EC)

11. Potential marketing authorisation holder (MAH) or other stakeholder interactions

What contacts /discussions (if any) have you had with a (potential) MAH of the active substance or other stakeholders (e.g. patient organisations, professional associations, research organisations, trade associations).

12. Any points the Applicant/Champion wished to address / expected benefits from the pilot

I agree that my candidate project submission is shared within the regulatory network.

List of Annexes

References

Annex – Academia status

Academia or academic sector, as defined in the above footnote, which is not financed or managed by private profit organisations in the pharmaceutical sector ("PPO"), nor has concluded any operating agreements with any PPO concerning their sponsorship or participation to the specific research project for which a fee reduction is sought for scientific advice. This should be evidenced by:

- (a) The Legal Entity Form (LEF) and the "founding document" (or any other suitable document provided during the application process).
- (b) Evidence should be provided of the place of legal establishment, which may be evidenced by the founding document or any other suitable document proving that the entity's seat is located in the EU, Iceland, Liechtenstein or Norway.
- (c) The applicant should not be under their direct or indirect control of any PPO in the pharmaceutical sector. Control may, in particular, take either of the following forms:
 - i. the direct or indirect holding of more than 50 % of the nominal value of the issued share capital in the applicant, or of a majority of the voting rights of the shareholders or associates of that applicant, or
 - ii. the direct or indirect holding, in fact or in law, of decision- making powers in the applicant.