Repurposing Pilot Project

Update and first insights

8th Industry stakeholder platform on research and development support – 11th July 2022

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Repurposing Pilot: Steps and Timelines

**Launch**
- 28th October 2021

**Pre-entry**
- Until 28th February 2022

**Selection**
- By 30th June 2022

**Scientific advice entry and outcome**
- EMA / NCAs SA timelines (Q4 2022)

**Filing of a new indication**
- Depends on uptake by an applicant/MAH

- Champions submit their candidate project(s) to the competent authority of their choice using the submission form

- Communication outcomes to the champions

Selection of candidate projects sufficiently mature to benefit from scientific advice (SA), in consultation with the EU-IN.
Phase 1 – Submission: first insights

35 candidate projects submitted to EMA

Therapeutic Areas

- ECV: 10
- IMM: 5
- INF: 1
- NEU: 15
- ONC: 4

Orphan Designation

- NO: 3
- YES: 32

Champions status

- Not-for-profit organisation: 17%
- Patient organisation: 3%
- Collaborative group: 9%
- Academia: 71%

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Step 2 - Selection phase: based on information provided in the submission form

Repurposing pilot project for authorised medicines

Submission Form
(To be submitted for up to three times to the OOA on repurposing pilot project – not of note for submission to EMA; please useitalics)

Active substance(s)
Provide the name(s) of the active substance(s) that are the subject of repurposing

Champion
Champion
Contact details
Provide contact details of the contact person (e-mail address, phone number...)

New therapeutic use targeted
New proposed condition or indication
Describe

Is the proposed new condition or indication for the authorised active substance(s) listed in section 4.4 of authorised medicinal product(s) in the EMA?
Yes/No

Do you hold an orphan designation for the proposed repurposing project?
Yes/No

Authorised medicinal product(s) in the EU/EEA
Authorised indication(s) (section 4.4 SPC)
To be listed/summarised

Authorised pharmaceutical form(s)
Section 3 SPC or an extract of the Public Data From Article 57 database can be provided as an annex.

Authorisation date (date of first authorisation, MAA name)
Section 9 SPC or an extract of the Public Data From Article 57 database can be provided as an annex.

Has the innovator / lead leader authorised medicinal product been granted a marketing authorisation more than 5 years ago?
Yes/No
Is an authorised medicinal product(s) containing the reviewed active substance out of patent / supplementary protection certificate (SPC) protection, and data and market exclusivity periods?
Yes/No

Champion characteristics
Please indicate if you are a for-profit organisation as per the definition in footnote* Yes/No
Please indicate if you are a patient organisation as per the definition in footnote* Yes/No
Please indicate if you are a collaborative group* (If yes, please provide the composition of the group)
Yes/No
Please indicate if you are an Academic Institution as per the definition in footnote* Yes/No
For academic only
Is the entity seated located in the EU, Iceland, Liechtenstein or Norway?
Yes/No
Are you meeting criteria c) of the Annex “Academic status”
Yes/No

Note: Of note, this information is of high applicability in academia for an orphan development but is without prejudice of taking part in the repurposing pilot

Applicant’s planned approach for scientific advice

Note: Only one pathway should be followed, either EMA or NCA

National Competent Authority (NCA)
Yes/No

Name of NCA
Please provide the name of the NCA

EMA
Yes/No

* Non-profit organisations and non-profit legal entity should be understood as a legal entity which by its legal form is non-profit oriented, which has a remit of not making a profit, or a legal entity which is understood as such in the state law, tax law or international law, which has legal personality and which shall, owing to its specific objectives, be exempt or partially exempt from tax obligations.

** Patentreorganisation should be understood as for-profit organisations which are patent focused, which is achieved by applying various strategies and may be in addition represented by patent agents and for-profit trade mark agencies.

*** Academic organisations should be understood as consisting of public or private higher education establishments that conduct research and education activities at the highest level of excellence and that grant academic degrees. These organisations are understood as entities of EU or NCA".

Authorised medicinal product(s) should be understood as medicinal products authorisation or authorisation by virtue of a marketing authorisation that has been granted by a competent authority in the Member State of the European Economic Area (EEA) or a national competent authorities under the scheme of the National Competent Authorities (NCA).

Submission Form
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The following elements are important to evaluate the plausibility and feasibility of an adapted medicine repurposing approach. Box sets contain minimum pieces of information that are 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 medicine(s) if applicable)

2. Proposed new condition(s)/indication(s)
   - Please consider as much as possible one or all of the components in the following mockup which may be relevant to cover in the targeted indication:
     - Description use & treatment(s) (preventive) or (symptomatic, curative or disease modifying if applicable) & (treatment of) (severity criteria if applicable) & (target disease or condition) & (target group) & patient(s) (restrictions to patient population, if applicable) & (pre-existing in terms of therapeutic option or prior therapy, or other restrictions, if applicable) & (in combination with other medicinal products (list relevant combination, if applicable) & (monotherapy))

3. Background information on the disease/population targeted and the unmet medical need
   - Background of the disease (including seriousness, population, prevalence, etc.)
   - Discussion on the current available treatment(s), with their limitations and disadvantages (reference to clinical guidelines could be useful)
   - 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 phases may have more reference 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, non-clinical data may be more thorough 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 pathology, different mode of action, different targeted population)
     b) Preliminary pre-clinical/clinical data / strength of current evidence to address the unmet medical need
     c) Final world data available (post-authorization studies, registry data, named patient basis, registration approval, off-label use)
     d) Indication is included in clinical guidelines or other recommendations such as health technology assessment (HTA)

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, GCP 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
    - c) Potential for Reactor use marketing authorization (RMA)
    - d) Potential for 1-year data exclusivity for a new indication (Article 10(5) of Directive 2001/83/EC)

11. Potential marketing authorization 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 organizations, professional associations, research organizations, 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.

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Repurposing Pilot: Next Steps

- Communication outcomes to champions – Q3 2022
- SA Pre-submission meeting with selected champions – Q4 2022
- Start of Scientific Advice – Q4 2022
Any questions?

Further information

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