EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Update on EU repurposing pilot

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Background of the EU Repurposing Pilot

focuses on the process of **facilitating data generation and/or data gathering** in accordance with regulatory standards to enhance the regulatory recognition of **new indications** for **well-established, authorised medicines**

aims to provide a visible **support to a not-for-profit stakeholder** (academia, patient organisations, collaborative groups and European References Networks), termed Champion.

To **help champions** present their proposed repurposing project **to regulatory authorities** and **seek advice**

Proposal for a framework to support not-for-profit organisations and academia (institutions and individuals) in drug repurposing

Prepared by a working group of the Safe and Timely Access to Medicines for Patients (STAMP) expert group

EU Repurposing Pilot

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HMA
Heads of Medicines Agencies

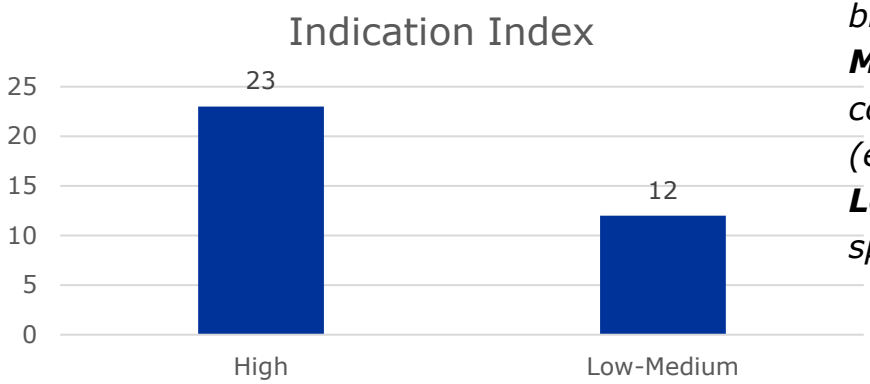
Launch	Pre-entry	Selection	Scientific advice entry and outcome	Filing New indication
28th October 2021	Until 28th February 2022	By 30th June 2022	Depends on respective EMA / NCA SA process	Depends on uptake by an applicant/MAH

Eligibility criteria considered in the context of the pilot

- Active substance with valid authorisation in the EU
- Out of patent / SPC / Data protection
- Applicants meets champion definition
- Is the new indication in a different condition than authorised indications
- Potential benefit to public health
- Level and quality of the preclinical/clinical evidence
- Availability of the medicines in the EU
- Champion located in the EU
- New indication well-recognised in clinical guidelines / scientific societies
- Evidence of extensive off-label use
- Ability by champion to conduct further research
- Likelihood of submitting an application

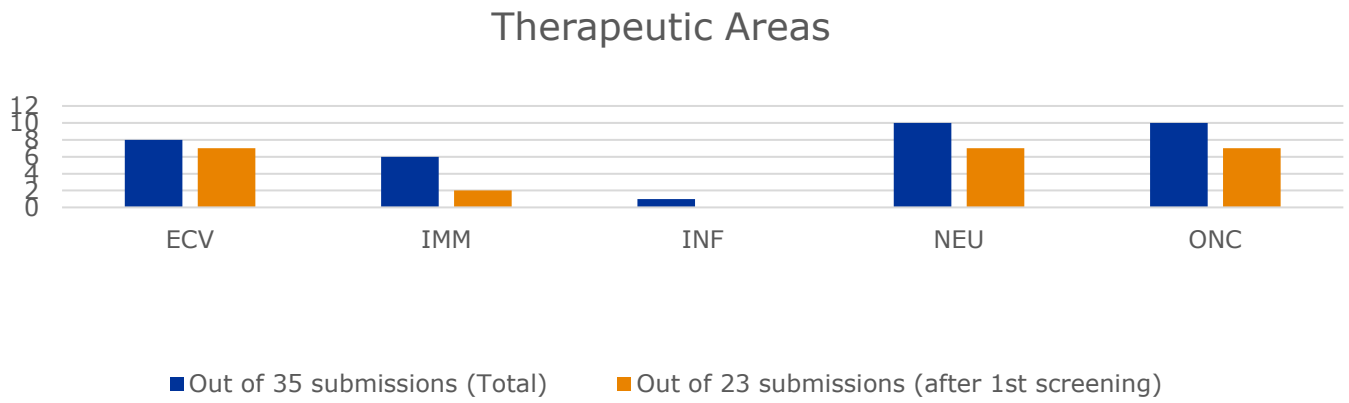
Some insights of EMA candidate repurposing projects (1)

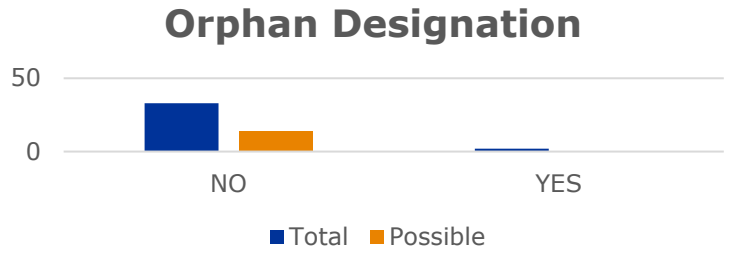
- 35 candidate projects submitted to EMA
- 23 projects targeting an indication in a different condition than the authorised indication



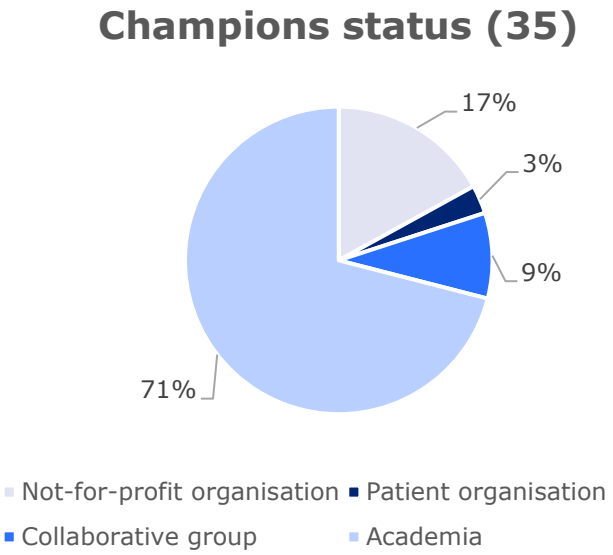
High *means a completely different new indication in a different condition than the authorised indication e.g. a bronchodilator in a neurological disease;*
Medium *may include new indication (in a different condition) but based on a common mechanism of action (e.g. anti-inflammatory in an inflammatory condition);*
Low *represents a new indication but in the same spectrum of disease.*

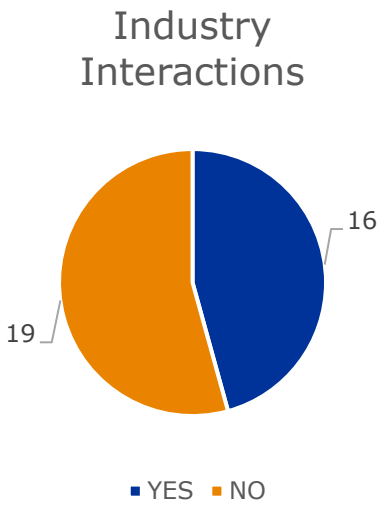
Some insights of EMA candidate repurposing projects (2)

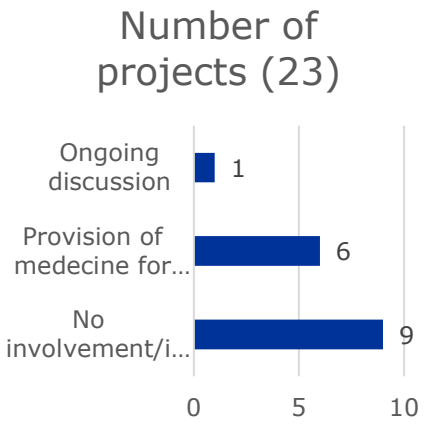




Some insights of EMA candidate repurposing projects (3)







Repurposing pilot project – tailored SA

- Ongoing scientific Advice for selected candidates receiving fee waiver

EMA informal meetings (Academia, RA, SA, RWE, Stats) with selected champions

SA Preparatory meetings with selected champions

Start of Scientific Advice procedures

Discussion meeting with SAWP and/or debriefing meeting after SA outcome

Consider early on any (potential) knowledge gaps or areas for which **RWD studies** could be helpful to support the SA, e.g. drug utilisation studies.

Few observations

Challenges :

- in expectation from the SA i.e. advice on programme development / study design, vs pre-assessment of data
- in understanding the SA, MAA / variations regulatory requirements and pathways to develop the regulatory strategy for the development and filing
- in securing the programme development due to challenges to find funding, hence this could result in a phase 3 not possible or jeopardised by off-label use.
- challenging in finding MAHs supportive to engage in a project

Opportunities:

- to enhance interactions with regulators
- get a better understanding of the regulatory and scientific requirements (discussion on choice of endpoint, validity of endpoint, RWD collection and study, type of MAAs, etc)

Next Steps

- ❑ To complete SA procedures for all selected projects (up to Q1 2024)
- ❑ To conduct survey to collect feedback from champions (up to Q1 2024)
- ❑ To publish report on EU repurposing pilot (targeted Q1-Q2 2024)

References

STAMP proposal for a repurposing framework
https://ec.europa.eu/health/system/files/2021-10/pharm773_repurposing_annex_en_0.pdf

Q&A on repurposing pilot project
Proposal for a framework to support not-for-profit organisations and academia in repurposing authorised medicines (europa.eu)

Submission form:
Submission form - Repurposing pilot project for authorised medicines (DOCX/138.84 KB) (new)

EMA scientific advice webpage
https://www.ema.europa.eu/en/human-regulatory/research-development/scientific-advice-protocol-assistance#scientific-advice-on-medicine-repurposing-(new)-section

Thanks to Anna Tavridou, Maribel Rico-Salas, Gregoire Borg

Any questions?

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