

Repurposing pilot report

PCWP/HCPWP Meeting –

19 November 2025

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Affairs Officer)



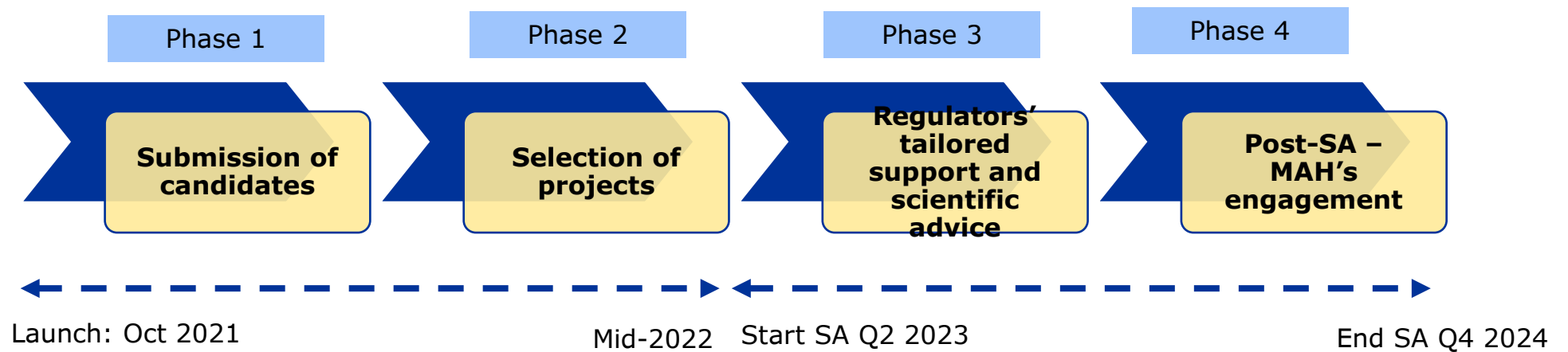
Background of the EU Repurposing Pilot



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Multistakeholder group: Inc. Member States, EMA, not-for-profit organisations (Eurordis, ACF), patients, industry (EFPIA, Mfe), HTA

Pilot phases



Choice of the champion to submit candidate projects either to EMA or volunteering NCAs to seek SA (free of charge)

Objectives of the pilot

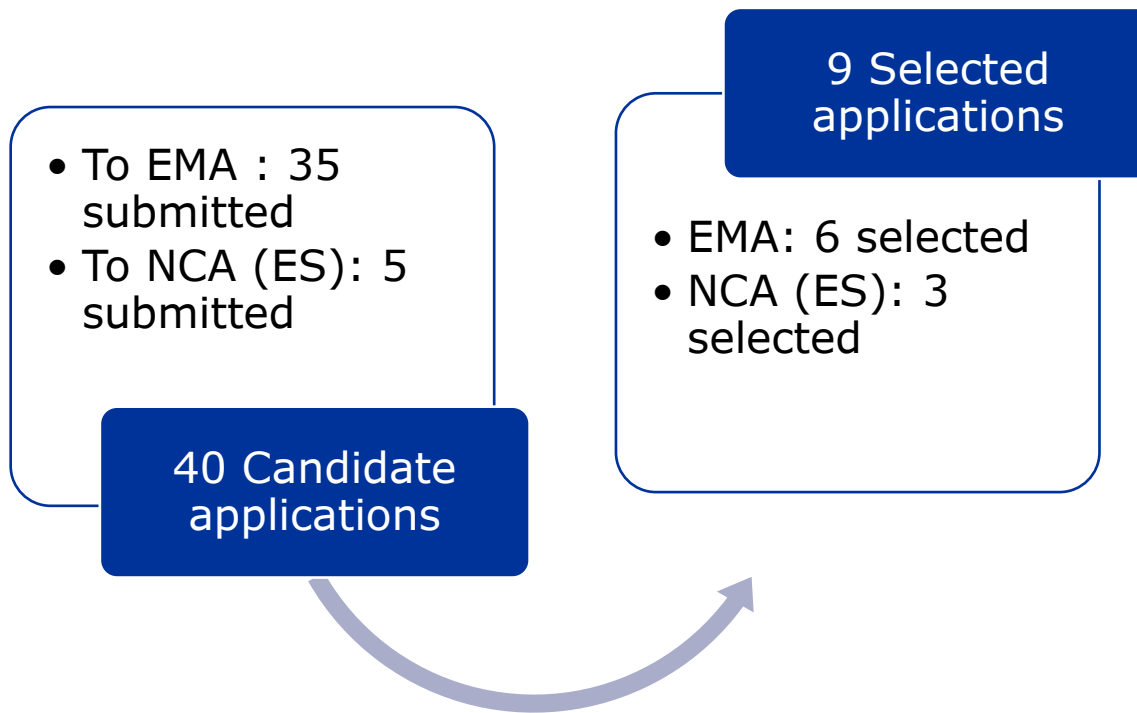
Identification and
characteristics of
repurposing projects in
pilot

Regulators' tailored
support

Added value of SA

Exploratory
(EMA RWE pathways &
MAH's engagement)

candidate projects



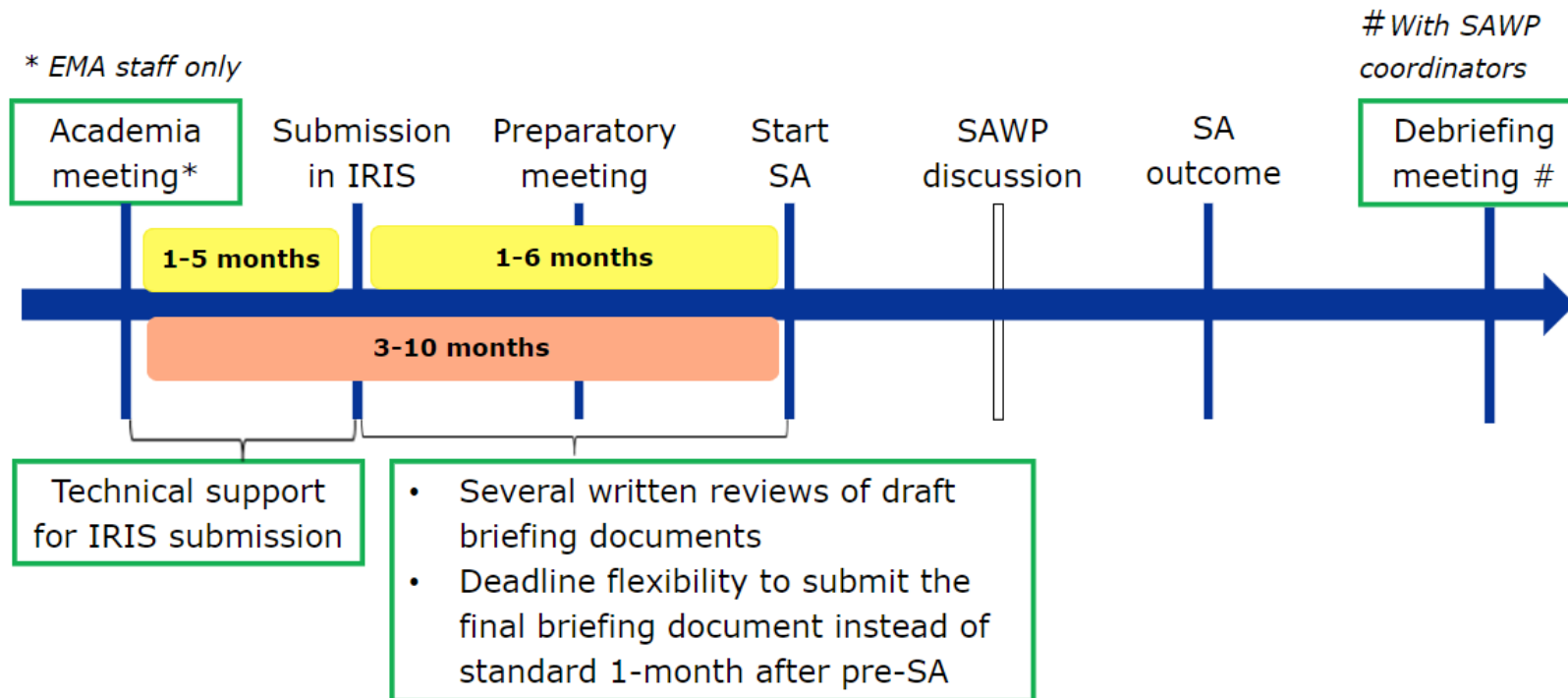
Mainly projects targeting **new indication in rare disease** (6 out of 9 selected),

led **by academic physicians**.

Various therapeutic area targeted,

5 projects concerned development also **in paediatric patients**.

Regulators' tailored support



Tailored regulatory support

Customised regulatory meetings and support

Scientific advice: topics & outcome

Selected projects included:

- **“Mature” dataset:** no plan for further development
- **Ongoing development:** at various development stages

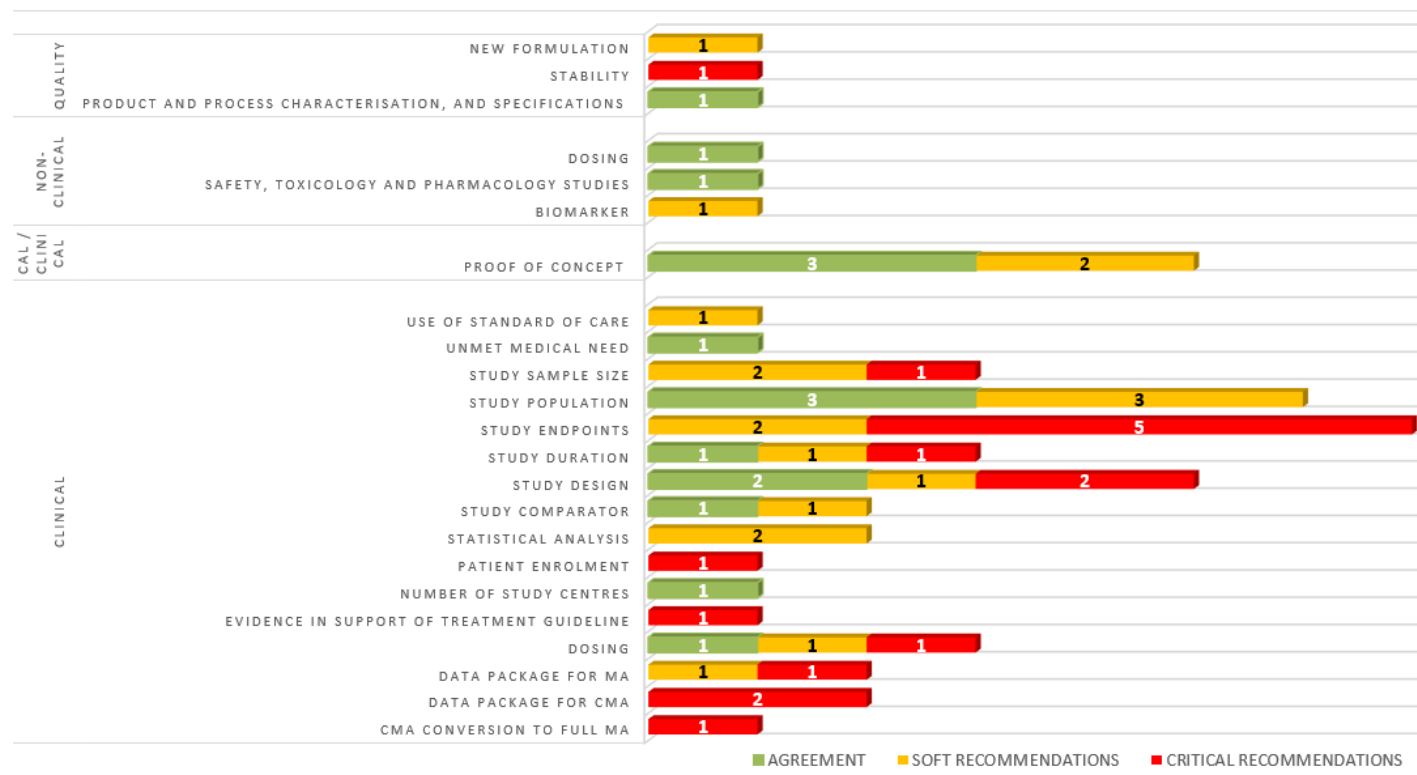


Figure 12: Frequency of agreement between champions' proposals and SA outcome per topics

the numbers in bars do not reflect the number of questions in SA but the topics of discussion (for instance a few questions related to the same topic were grouped).

Scientific challenges

Defining patient populations, meaningful endpoints, dosing, and isolating investigational effects posed scientific challenges.

Key lessons learnt

Tailored regulatory support

Customised regulatory meetings were essential but required significant resources from regulators and champions.

Importance of regulatory support

The pilot highlighted the critical role of regulatory guidance in advancing repurposing projects and need to seek advice in early development phase.

Limited industry engagement

Pharmaceutical companies showed minimal interest in new indications due to commercial and regulatory complexities.

Need for ecosystem support

Broader support such as funding, partnerships, and streamlined pathways is crucial for successful drug repurposing.

Regulatory outcome

1 project achieved on-label approval during the pilot

What's next?

1. Projects selected within the pilot

- Continued **regulators'** support including **follow up scientific advice**

2. Future repurposing projects - Considerations to support data gathering and generation

- **Not-for profit organisations to consider seeking SA and engaging dialogue** with regulators (EMA and NCAs) **early in development phase**
- **Suite of measures as relevant specific to the project** e.g. ad hoc interactions; review of briefing materials; debriefing and facilitating meetings with Coordinators and Rapporteurs, as appropriate
- **Increasing RWE footprint into repurposing activities** when needed

2. Future repurposing projects - Considerations on uptake by MAHs

- Industry Associations encouraged to develop ways to help not-for profit organisations to reach out to their members companies
- EMA and NCAs can **explore closed and open multi-stakeholder interactions, as appropriate**
- **New Pharma legislation – upcoming policy changes** aimed at supporting repurposing



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Thank you

EMA: Anna Tavridou, Gregoire Borg, Ralf Herold, Francesco Pignatti, Jane Moseley, Stiina Aarum, Milton Bonelli, Maria Tome, Iordanis Gravanis, Thomas Girard, Maribel Rico-Salas, Alexandra Pacurariu, Kelly Plueschke, Stefanie Prilla, Theodor Framke, Inga Abed, Caroline Voltz

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