DRAFT Proposal for a Repurposing Framework

EFPIA and Medicines for Europe

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European Federation of Pharmaceutical Industries and Associations

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Framework for Repurposing* – Key elements to consider

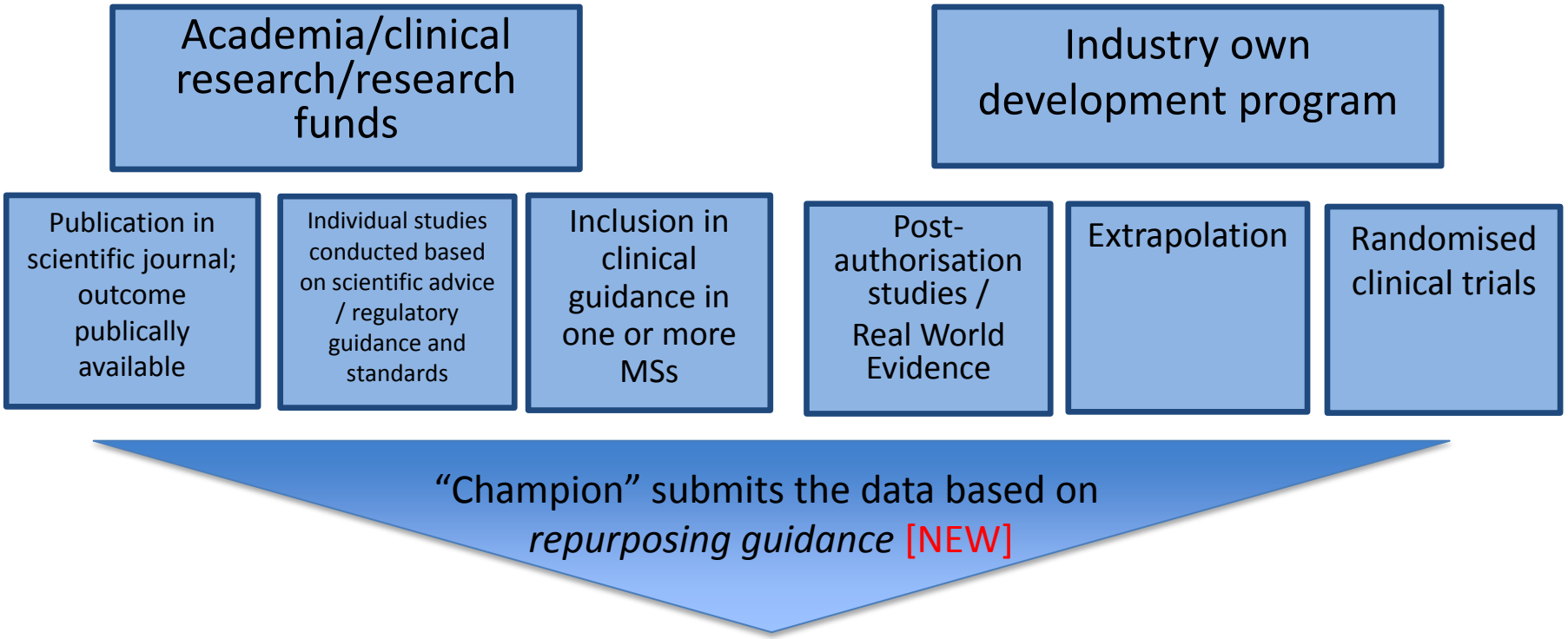
For a <u>new use</u> for an <u>off-patent compound</u>

- 'Champion**' puts forward a <u>repurposing proposal</u> for regulatory assessment
  - Standard format/package (guidance provided by regulators)
    - Compound (or product if it exists)
    - Proposed repurposing (prevention, treatment or diagnosis of disease)
    - Supporting data for indication
- <u>Regulatory evaluation</u> decides whether proposal is supported:
  - Standard evaluation (based on existing EC/EMA guidances -> *repurposing procedural guidance* is needed)
    - Scientific rationale for repurposing
    - Status of proposed indication (unmet need, population, etc)
    - Eligibility of data
- If assessment is positive, it is made available in a '<u>repurposing Data pool</u>'
  - Possibility of partnership of 'champion' with MAHs or other interested parties to pursue a repurposing opportunity

*In addition and in alignment to already existing regulatory options

**Champion can be a person/academia/research fund/company with a particular interest in repurposing a product for a new indication

Data Sources



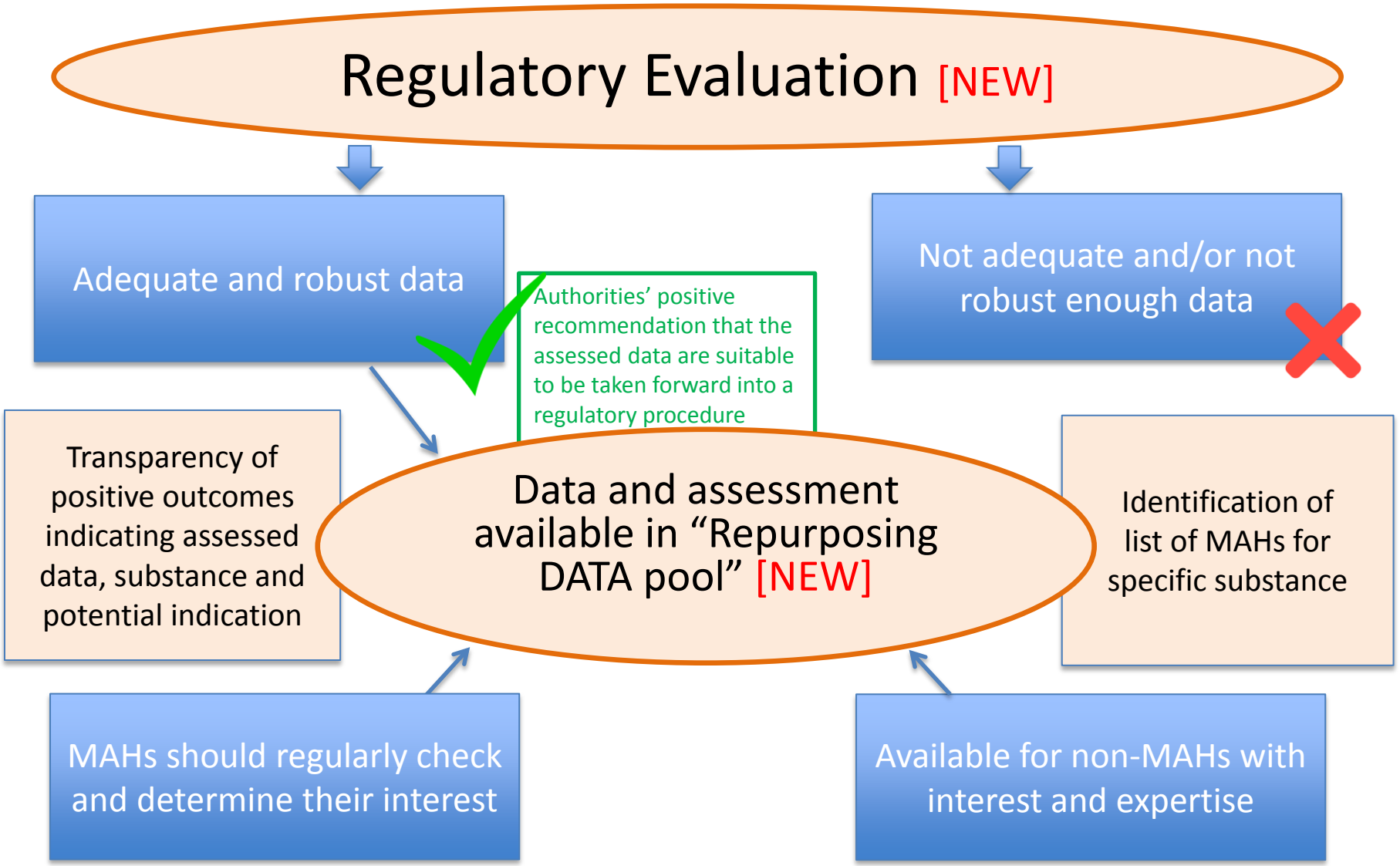
Conditions for Eligibility* of Data – Regulatory Evaluation

To be considered by the regulatory evaluation [NEW], e.g.:

- Is there a scientific rationale?
- Is there an unmet medical need?
  - No licensed treatment
  - Severity of condition
  - Supply considerations
  - Access and affordability
- Is there a specific need for a subset population/disease?
  - Children
  - Rare disease
  - Geographic needs (specific needs for certain countries)
- Is the data robust from efficacy and safety perspective?
  - Studies / publications not used for regulatory dossier before

*In the view of limited resources, the listed criteria can be used for prioritisation.

From Regulatory Evaluation to "Repurposing DATA Pool"



Data Adequate and Robust

- In some cases the data available for a possible new indication is "ready for submission"
  - No post-authorisation commitments required
  - No new formulation nor dosage form
- Assessing regulator(s) to facilitate the discussion to agree on next steps
  - Utilisation of an existing regulatory pathway (as streamlined as possible), e.g.:
    - New MA for orphan drug, PUMA
    - Addition of indication in existing product(s) through (simplified) variation pathway:
      - » Type IB variation if data already assessed by the regulators and only PIL/SmPC amendment needed
      - » Type II variation if data still needs to be assessed to some extend by the regulators
  - Consideration of risk management and pharmacovigilance aspects
- Additionally, specific consideration of appropriate incentive(s) and impact on pricing is needed

Data not Adequate nor Robust  *- what could be done?*

- In most cases (ref. cases studies shared with STAMP) the data available for a possible new indication is not “ready for submission”
  - New studies required; safety and/or efficacy
  - Post-authorisation commitments required; safety and/or efficacy
  - Different formulation and/or dosage form need to be developed for specific population/indication needs
- Consider making data and assessment available in any case to the Repurposing DATA Pool
  - Facilitate the possibility for MAHs/non-MAHs to express interest in taking data forward
  - Facilitate the transparency of regulatory assessment
- Consider bringing the interested MAH(s) together to discuss the next steps
  - Set up a Public/Private Partnership consortium to build the needed infrastructure, create the guidance and practice the ways of working in order to take forward some real test cases
  - Address the limitations of the data and/or current product dossier
  - If non-MAH involved, facilitate collaboration with existing MAH(s)
  - Outline the use of existing regulatory tool/pathway
- Consider provisions of already existing Regulations to support further development
  - Paediatric Regulation 1901/2006 Art 40: Funding of studies into off-patent medicines for children (‘MICE’)
  - Orphan Medicinal Products Regulation 141/2000 Art 9: Incentives made available by the Community and by the Member States to support research into, and the development and availability of, orphan medicinal products and in particular aid for research for SMEs undertakings provided for in framework programmes for research and technological development.
- Consider incentives needed to conduct the further work
  - Remove disincentives, such as promoting economic off-label use
  - Free scientific advice in the course of repurposing pathway and/or decreased/waived fees for variations could be new incentives

Summary of the DRAFT Proposal for a Repurposing Framework