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SCIENCE MEDICINES HEALTH

Strategy and pilot phase for patient registries

Draft – still under discussion

Industry Stakeholder Platform - Operation of EU Pharmacovigilance Legislation
12 January 2015

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Problem statement

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- Registries are requested to MAHs in the context of risk management plans and as regulatory requirements, e.g. for advanced therapies, medicinal products for paediatric use and orphan products.
- The current approach to registries is sometimes suboptimal in scientific and resource terms:
  - existing disease registries are not fully exploited, which may lead to duplication of efforts and inefficiencies
  - lack of common protocols, scientific methods and data structures
  - lack of data sharing and transparency
  - lack of sustainability
- Difficulty to assess the validity of results from individual registries
- On-going national and EU initiatives on registries not well coordinated.

The Regulators' dilemna

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- *Disease registries* are preferred to product registries in that they gather insights in clinical aspects of conditions rather than in outcomes of specific treatments and allow comparisons.

- Regulators may impose initiation of *product registries* – disease registries are more difficult to put in place and are often integrated in a national or regional health care system.

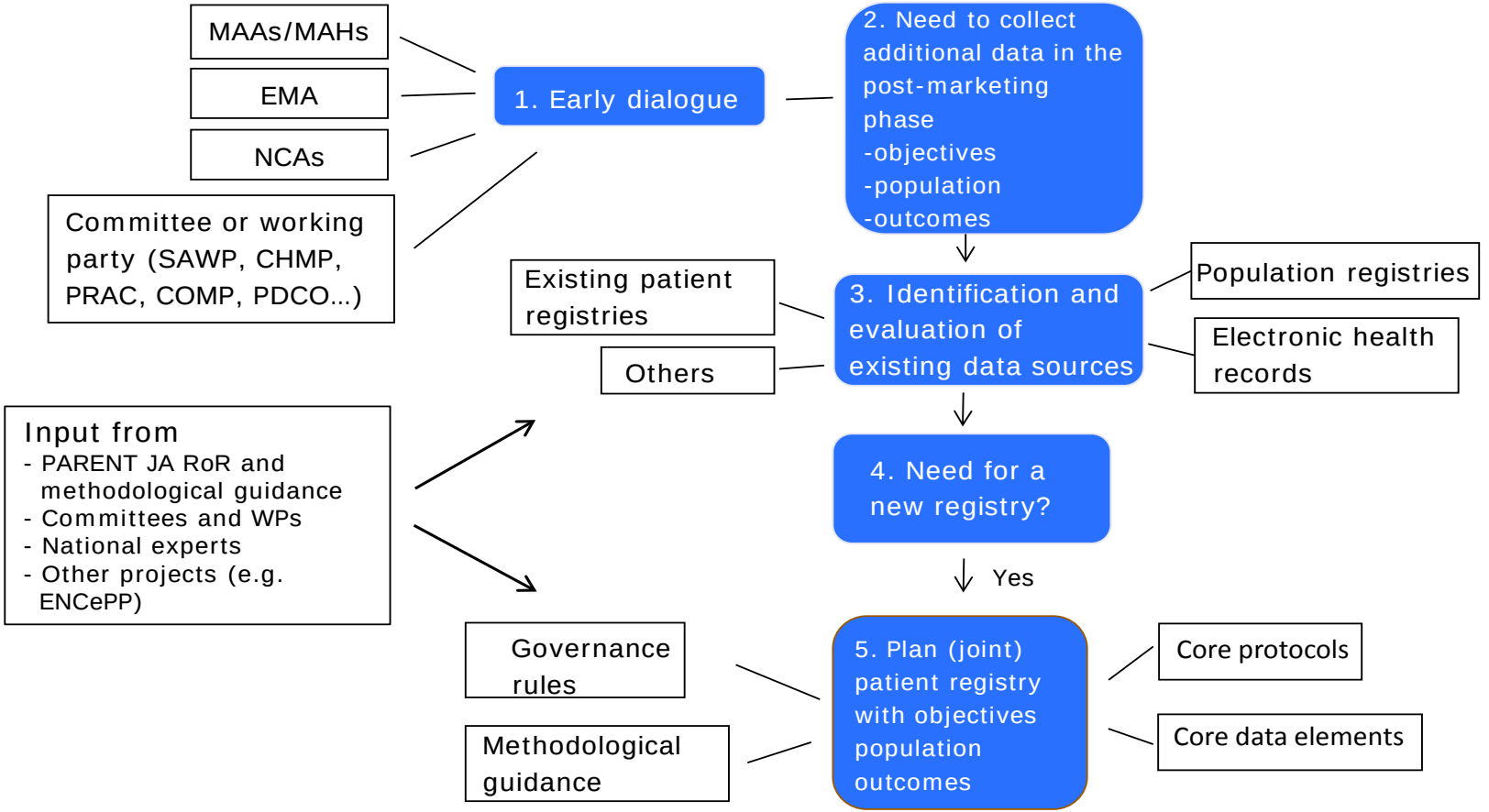
Patient registries

Registry: organised system that uses observational methods to collect uniform data on specified outcomes in a population defined by a particular disease, condition or exposure.

- Registries to be defined by their objectives rather than a-priori decision about recruitement strategy
  - ➢ Choice of population, outcomes and methodology to be defined in relation to the objectives

- Methodological standards common to all types of registries
- Many registries include treated patients
- Some governance issues linked to industry-sponsored registries can be solved

Proposed approach



Opportunities

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- EMA scientific advice procedure and pro-active preparation of risk management plan allow early effective dialogue between MAA, committees and NCA to discuss objectives, outcomes and methodology of registries pre-authorisation
- New pharmacovigilance legislation provides legal mandate for EMA and NCAs to impose/support registries and encourage joint studies.
- Joint Action on Cross-Border Patient Registries iNiTiative (PARENT JA): draft methodological guidance and core data elements for registries
- future Joint Action II on registries
- Other EU projects: European Reference Networks (ERN), RD CONECT (integrated platform for registries and biobank), European Research and Infrastructure Consortium (ERIC) platform for registries, JRC project for medical devices, European platform of rare disease registries, other disease registries (eg network of European cancer registries)
- National registries

EMA's proposal

- Cross-Crommittee Task Force established to finalise strategy paper and support further steps
- Identification/development of tools
- Proposal for Pilot phase of EU Collaborative network

Primary objective

to develop and test an EU collaborative framework for patient registries that would facilitate the collection and analysis of high quality data to inform regulatory decisions and the benefit-risk profile of medicinal products.

Long term objective

to test the feasibility of integrating registries in the adaptive licensing pilot, the one-stop shop strategy and the joint discussions between regulators and HTA bodies/payers.

What will be delivered by the pilot phase?

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- Strategy paper with rationale, criteria for disease/product selection, roles and responsibilities, methods and timelines for pilot phase.
    - methodological challenges: eg. optimum use of registries, linkage to other data sources
- Technical specification based on PARENT JA and other initiatives including standard methods and processes for patient registries:
    - core elements of a standard protocol and common data elements
    - components of a standard methodology and governance principles
    - guidance on data privacy rules applicable to the registry data and their access rights.
- Results of the pilot phase on 2-4 patient registries, lessons learnt and areas for improvement.

What will NOT be delivered by the pilot phase?

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- Criteria for deciding whether additional data collection is needed or not, and its objectives
- New methodological guidance and data elements (PARENT JA outputs will be used)
- IT tool for collecting and pooling data from different registries.

How best to engage with industry?

Further information

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