

5 May 2017  
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Inspections, Human Medicines, Pharmacovigilance and Committees Division

## Patient Registry Initiative- Strategy and Mandate of the Cross-Committee Task Force

### EMA Initiative

#### 1. Background

Patient registries are organised systems that use observational methods to collect uniform data on a population defined by a particular disease, condition, or exposure, and that is followed over time. High quality patient registries can make valuable contributions to the evaluation and monitoring of medicines for public health benefit, especially in relation to their safety and notwithstanding that most registries were not established for this purpose.

Regulators and pharmaceutical companies currently face a number of challenges in using existing registries or establishing new ones to support medicines evaluations during the marketing authorisation process, including a lack of:

- coordination between ongoing initiatives at national and international levels
- harmonised protocols, scientific methods and data structures
- data sharing and transparency
- long-term sustainability of registries.

These factors have led to under-use of existing patient registries, inefficiency when registries are used, non-useable registries, and a duplication of efforts. To address these problems, and given the importance of harnessing real world data to support benefit-risk monitoring of medicinal products, the EMA initiative seeks to create a European Union-wide framework on patient registries, facilitating collaboration between:

- registry coordinators including healthcare professionals' associations, patients' associations, academic institutions or national agencies responsible for overseeing healthcare services
- potential users of registry data, such as medicines regulators, reimbursement bodies, and pharmaceutical companies.

There is a need for common approaches to foster the optimal use of national and multinational registries and there is an opportunity for EMA together with the Committees to further this activity.



## **2. Why the Patient Registry Initiative is important**

### **A. Benefit-risk evaluation**

- Patient Registries provide observational data that may contribute to medicines benefit-risk evaluation during the marketing authorisation process and post-authorisation depending on the disease area.
- In certain areas, small numbers of patients and/or events place complex demands on medicines evaluation and randomised controlled trial data may be limited or unfeasible to collect. In these situations, the Initiative should be supportive in gathering high quality, harmonised registry data that may contribute to the evaluations.

### **B. Registries Network**

- There are few formal networks currently of patient registries to permit regulatory and/or pharmaceutical company stakeholders to identify relevant patient registries.
- At present, when registries are used (or considered for use) in post-authorisation studies, there is inconsistent communication between regulators, registry holders and marketing authorisation holders.
- An inventory of patient registries will help to address the foregoing shortcomings by assisting the development of EU-wide networks that promote harmonisation of data collection in different disease areas and between-registry interoperability.
- The Patient Registry Initiative supports the collection of standardised core data sets in individual disease areas across the European Economic Area with transparent consents, governance and data sharing agreements between the stakeholders.

## **3. Aims**

The Patient Registry Initiative has at its core the following key aims:

- To facilitate impartial discussions at an early stage in the authorisation procedure to increase use of existing patient registries
- Where no suitable registry exists currently to support an authorisation procedure, to facilitate where possible the creation of a new registry based on standard methodological approaches to ensure wider downstream applicability.

Annex 1 includes the vision for the Patient Registry Initiative

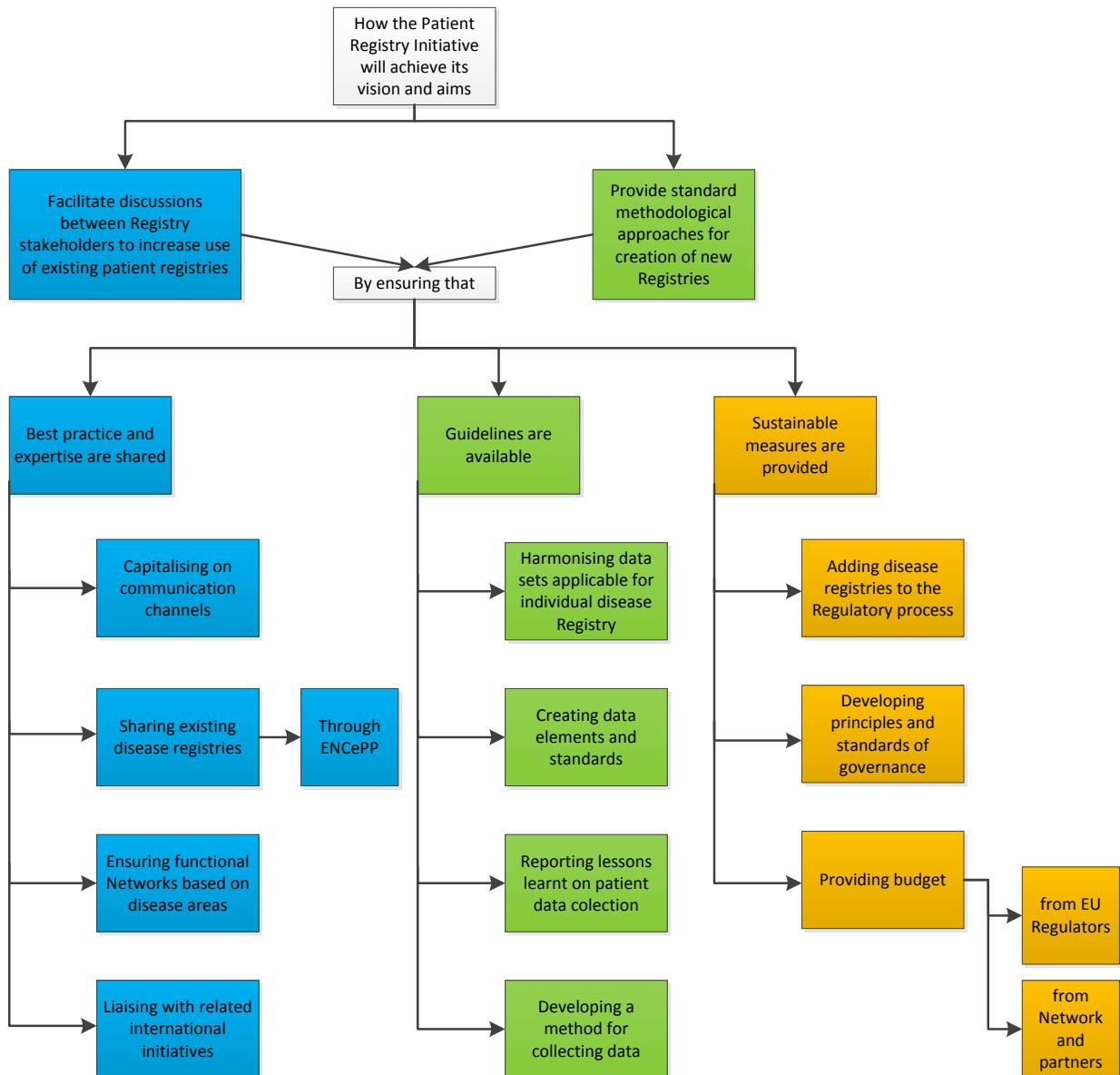
## **4. How to achieve the Patient Registry Initiative vision**

Based on the current challenges and factors highlighted in the Background section, the Patient Registry Initiative has developed and agreed on a vision for Patient Registries based on three objectives:

1. To protect public health through better use of registry data enabling post-authorisation studies to support benefit risk evaluation

2. To facilitate the harmonisation of data collected by individual disease registries
3. To make better use of networks of patient registry stakeholders

The Figure below summarises the strategy to achieve the vision and the aims of the Patient Registry Initiative.



#### 4.1. Objectives

- a) To protect public health through better use of registry data enabling post-authorisation studies to support benefit risk evaluation
  - ✓ Communicate the Patient Registry Initiative to individual EMA committees to raise awareness on the consideration of registry data where appropriate in the marketing authorisation process. Propose objectives in Committee workplans;

- ✓ Qualify registries that have demonstrated appropriately high quality data collection and governance;
- ✓ Embed registry questions at scientific advice pre-submission/validation, day 120, and day 180 time-points. Work with EMA committees to further develop a framework whereby, throughout the authorisation process, EMA and assessors consistently consider if appropriate registry data are available and if authorisation applicants should be advised to consider incorporating them.

b) To facilitate harmonisation of data collected by individual disease registries:

- ✓ Bring registry, regulatory, patient, pharmaceutical, reimbursement, academic and clinical stakeholders together
  - i. to discuss their common data needs and agree on core data elements that could feasibly be collected by all registry holders
  - ii. to ensure appropriate consents and governance arrangements are in place to permit data sharing with stakeholders
  - iii. to encourage diseases registry holders to apply for qualification where appropriate
- ✓ Informed by the stakeholder meetings, create guidelines on i) harmonising data collection and interoperability, ii) establishing data quality standards, and iii) ensuring governance arrangements to permit data sharing that are generalisable to patient registries broadly
- ✓ In new product pipeline disease areas where no registries currently exist, provide EMA budget to facilitate stakeholder workshops that will inform 'operating models' for new disease registries, particularly where new waves of products for specific rare diseases are foreseen.

c) To make better use of networks of patient registry stakeholders:

- ✓ Assist stakeholders to identify existing registries by inviting registry holders to have their information and links hosted on ENCePP so that all stakeholders have a point of contact
- ✓ Use the ENCePP platform to share with stakeholders guidelines and examples of best practice in use of registry data and to facilitate between-stakeholder communications
- ✓ Make publicly available the reports and operating models arising from the stakeholder workshops
- ✓ Explore mechanisms to help sustain registries

## **5. Evaluation and review of strategy**

To measure the effectiveness of this strategy, Key Performance Indicators to be reviewed annually are proposed as follows:

1. Number of registries hosted on ENCePP
2. Number of workshops facilitated under the umbrella of the Patient Registry Initiative
3. Number of registries approaching EMA for advice on qualification
4. EMA Committees incorporating consideration of registries in their processes
5. Number of registries approaching EMA for advice on initiating common data sets
6. Number of Registry-based studies requested by Agency committees

## **6. Mandate of the Cross-Committee Task Force**

The mandate of the Cross-Committee Task Force is to:

- Develop and Lead the Patient Registry strategy
- Support the implementation plan agreed by the EMA and Committees
- Report on the progress and deliverables of the Patient Registry Initiative to the EMA and Committees and to communicate to stakeholders.

## **7. Membership and organisation:**

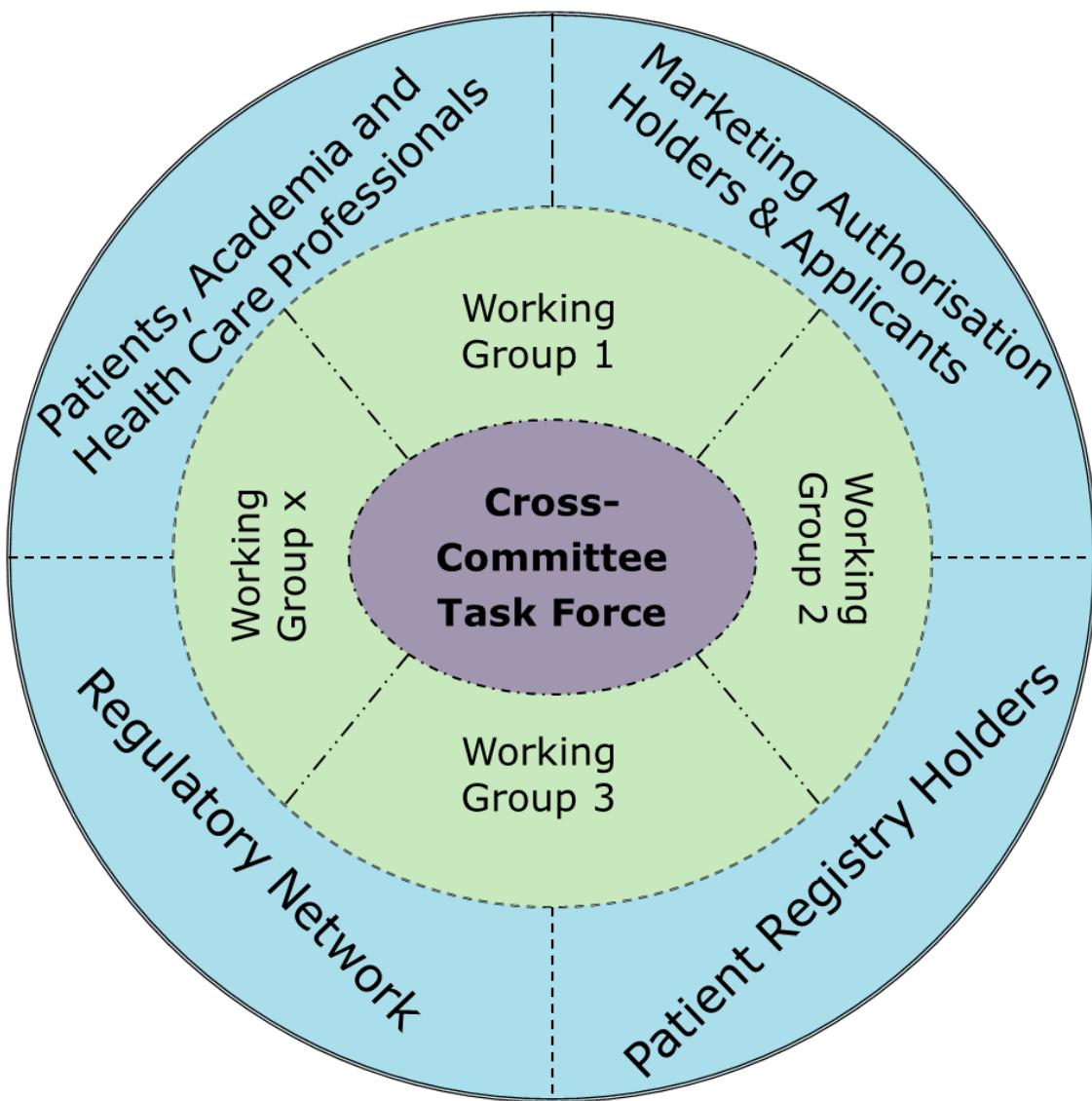
**Membership of the Cross-Committee Task Force:** Task Force members express their interest on a voluntary basis and are appointed by the co-chairs for a two year period based on the activities to be performed. The Committee for Medicinal Products for Human Use (CHMP) and the Pharmacovigilance Risk Assessment Committee (PRAC) are always represented. There is an annual review of membership to ensure it remains appropriate whilst ensuring adequate rotation. The Committee Task Force meets six times a year via teleconference or Adobe-connect. Additional meetings can be organised on an "as needed" basis.

**Cross Committee Task Force Working Groups:** To ensure the successful execution of the Cross Committee Task Force workplan, Working Groups are created as needed. A Working Group consists of a Chair and two to four members who together are responsible for delivering specific tasks agreed by the Cross-Committee Task Force. Additional members are appointed on a short-term basis according to expertise required for specific areas of activity. More than one working group may be created in parallel. The working group meets on a monthly basis via teleconference or Adobe-connect. Additional meetings can be organised on an "as needed" basis. The working group ceases when its goal has been achieved.

## **8. Related documents**

- Communication Strategy

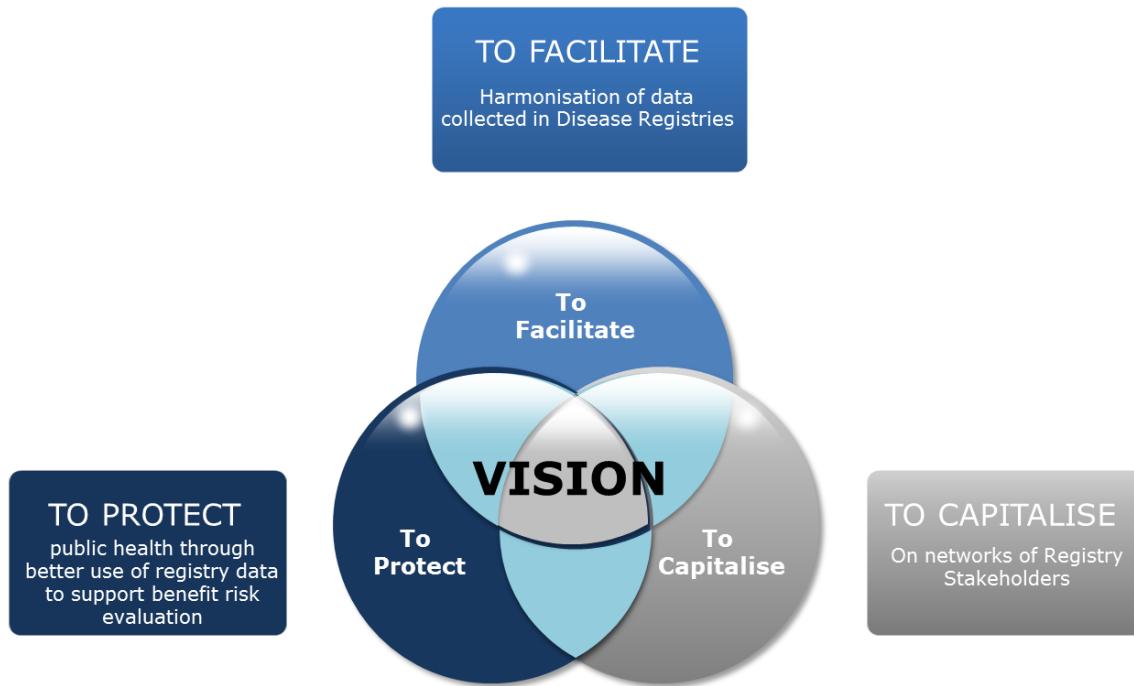
**Figure 1: Patient Registry governance structure**





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**ANNEX 1: Patient Registry Initiative Vision**



***Patient Registries Initiative***

30 Churchill Place • Canary Wharf • London E14 5EU • United Kingdom

**Telephone** +44 (0)20 3660 6000 **Faxsimile** +44 (0)20 3660 5520

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