EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Today

Workshop on the role of registries in the monitoring of cancer therapies based on genetic and molecular features

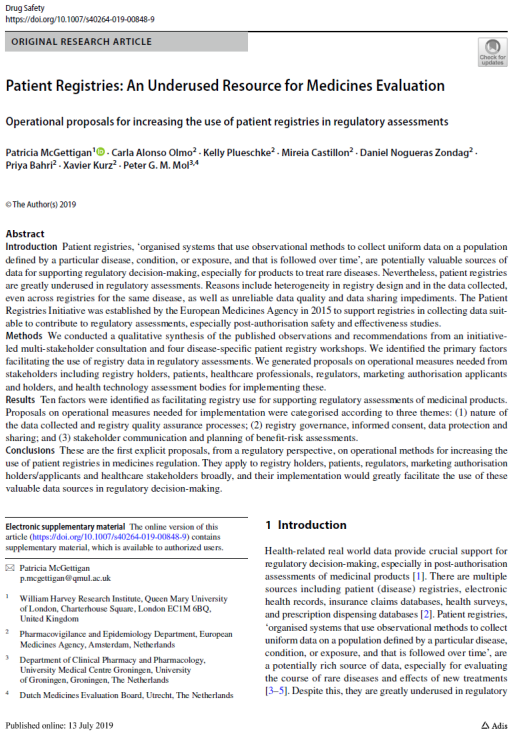
29th November 2019

Registries for monitoring of cancer therapies based on genetic and molecular features

Targeted therapies approved with remaining uncertainties

- Registries : "organised systems that use observational methods to collect uniform data on specified outcomes in a population defined by a particular *disease*, *condition* or *exposure*"

- Preference for patient (***disease***) registries:
  - clinical outcomes of conditions with different treatments, rather than outcomes of specific treatments
  - allows comparisons
  - Integrated in health care systems



Drug Safety
https://doi.org/10.1007/s40264-019-00848-9

ORIGINAL RESEARCH ARTICLE

Patient Registries: An Underused Resource for Medicines Evaluation

Operational proposals for increasing the use of patient registries in regulatory assessments

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Abstract

Introduction Patient registries, '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', are potentially valuable sources of data for supporting regulatory decision-making, especially for products to treat rare diseases. Nevertheless, patient registries are greatly underused in regulatory assessments. Reasons include heterogeneity in registry design and in the data collected, even across registries for the same disease, as well as unreliable data quality and data sharing impediments. The Patient Registries Initiative was established by the European Medicines Agency in 2015 to support registries in collecting data suitable to contribute to regulatory assessments, especially post-authorisation safety and effectiveness studies.

Methods We conducted a qualitative synthesis of the published observations and recommendations from an initiative-led multi-stakeholder consultation and four disease-specific patient registry workshops. We identified the primary factors facilitating the use of registry data in regulatory assessments. We generated proposals on operational measures needed from stakeholders including registry holders, patients, healthcare professionals, regulators, marketing authorisation applicants and holders, and health technology assessment bodies for implementing these.

Results Ten factors were identified as facilitating registry use for supporting regulatory assessments of medicinal products. Proposals on operational measures needed for implementation were categorised according to three themes: (1) nature of the data collected and registry quality assurance processes; (2) registry governance, informed consent, data protection and sharing; and (3) stakeholder communication and planning of benefit-risk assessments.

Conclusions These are the first explicit proposals, from a regulatory perspective, on operational methods for increasing the use of patient registries in medicines regulation. They apply to registry holders, patients, regulators, marketing authorisation holders/applicants and healthcare stakeholders broadly, and their implementation would greatly facilitate the use of these valuable data sources in regulatory decision-making.

Electronic supplementary material The online version of this article (https://doi.org/10.1007/s40264-019-00848-9) contains supplementary material, which is available to authorized users.

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1 Introduction

Health-related real world data provide crucial support for regulatory decision-making, especially in post-authorisation assessments of medicinal products [1]. There are multiple sources including patient (disease) registries, electronic health records, insurance claims databases, health surveys, and prescription dispensing databases [2]. Patient registries, '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', are a potentially rich source of data, especially for evaluating the course of rare diseases and effects of new treatments [3–5]. Despite this, they are greatly underused in regulatory

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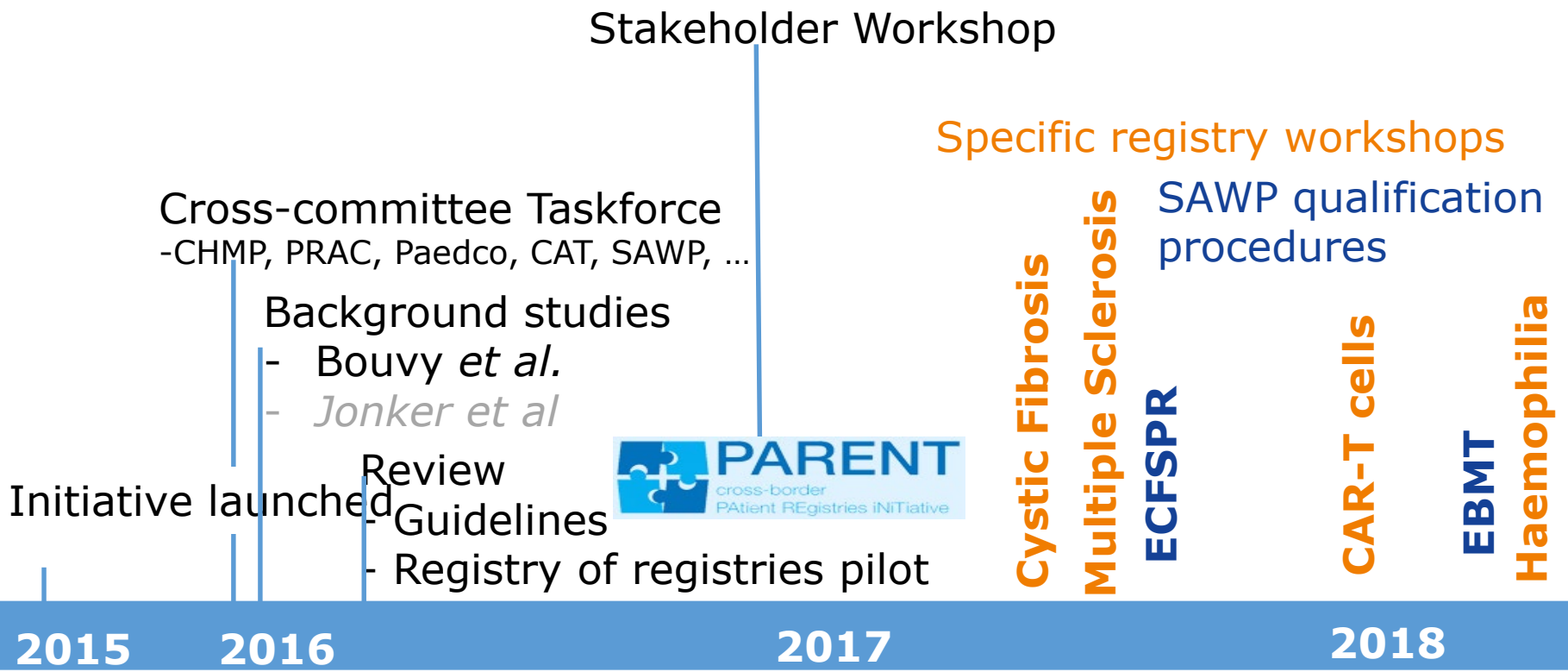
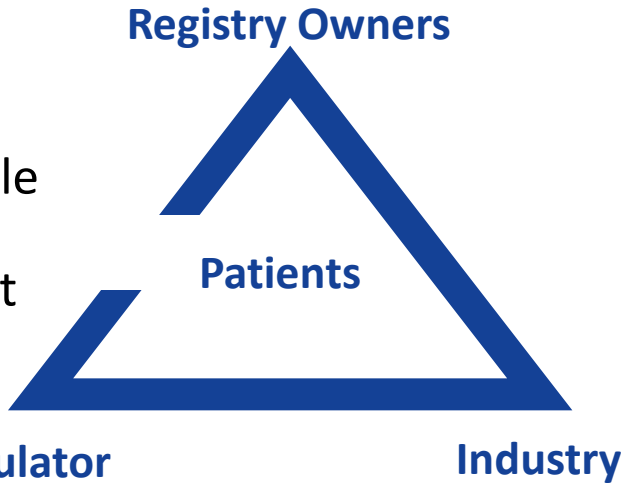
Patient Registry Initiative



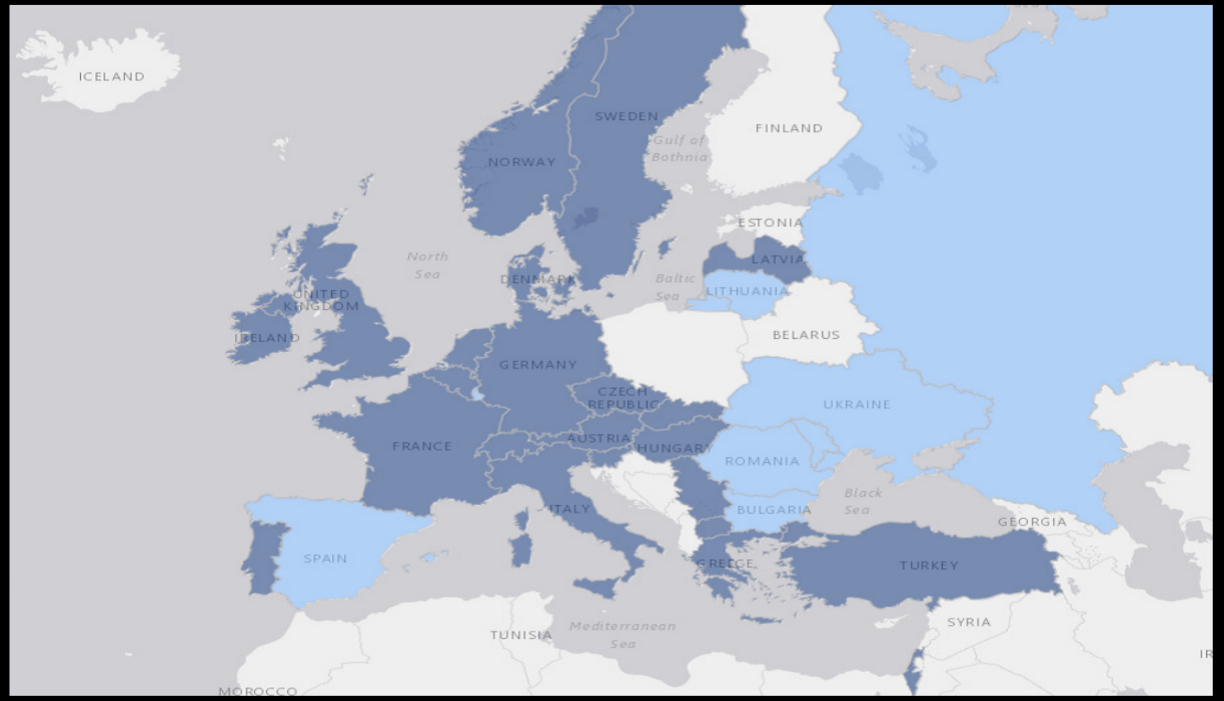
How can regulators support use of disease registries?

- **Methodological guidance** on use of disease registries from a regulatory perspective: forthcoming - Will address regulatory requirements and guidance for collecting / reporting AEs and ADRs
- **Scientific Advice** on PASS/PAES study protocol using registries, e.g. joint collaborative studies
- **Inventory of disease registries**
- **Facilitation of interactions** between regulators, industry and registry holders during the entire life cycle of a product
- **Collaboration with EU initiatives**, e.g., EUnetHTA Joint Action 3, EC JRC European Platform on Rare Disease Registration
- **Qualification procedure**



General Queries: Coverage of ECFS Registries

- 31 Countries
- **>42,000 patients**
- 17 National Registries
  - 12 Upload
- 85 centres use ECFS Registry Software*
- GPP not currently required
- Broad coverage



* + 13 centres of the CF Registry Ireland

Presented ECFS 2017

Procedure No.: EMEA/H/SAB/080/1/QA/2017
EMA/CHMP/SAWP/802259/2017
Product Development and Scientific Support Department

Qualification Opinion

The European Cystic Fibrosis Society Patient Registry (ECFSPR)

Context of Use

Drug utilisation studies

- For total recorded population and by subgroup such as CF complications, age, gender, FEV1 status, genotype, etc.

Drug efficacy/effectiveness studies

- For concurrent assessment of post authorisation efficacy/effectiveness using annual best FEV1, mortality, pulmonary exacerbations using the ECFSPR working definition or CF complications;

- As a source of historical control data ..for contextualization, e.g. for comparative purposes in the context of non-randomized clinical trials (i.e. when this would be the only reasonable option).

Drug safety evaluation

- As a tool to collect safety data with a particular focus on important identified and potential risks.

{and some fine print qualifications}

Today's program

Introductory talks

- Regulatory views on needs
- Landscape of existing registries
- Breakout sessions
  - ***Core data elements***
  - ***Data quality***
  - ***Governance***
- Plenary presentations
- Next steps
- *But, now first some housekeeping rules☺*

Health & Safety rules at the EMA

- Please wear your badge AT ALL TIME
- Grey and Red badges: You need to be escorted by a Blue badge to leave the floor
- Toilets are located on this floor
- For Lunch (13:00 CET):
  - ✓ Black and Grey badges can go to the EMA canteen (-2 floor) – wait in front of room 0-C to be escorted
  - ✓ Red badges should have lunch outside the EMA building - wait in front of room 0-C to be escorted, and at the EMA reception before meeting restarts

In case of a fire…

- Activate the fire alarm and Building Security will call the fire brigade
- On hearing the fire alarm: follow the instructions provided by the voice message:
  - ✓ Remain on the floor where you are (do NOT leave the floor you are on),
  - ✓ Be prepared to leave the building if necessary by the nearest available fire exit, DO NOT ATTEMPT TO USE THE LIFTS
  - ✓ Wait for further instructions which will be given by a floor marshal.

And:

- Participants in the room + remotely
- Therefore please use the microphone and present yourself before you speak

- Plenary presentations will be published on the EMA website after the workshop

Thank you for your attention

Further information

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