Patient Registries Initiative
Background, Achievements, Next steps

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ENCePP Plenary meeting

Presented by Xavier Kurz, Surveillance & Epidemiology Service, European Medicines Agency
Patient Registries

1. Patient Registries Initiative: Background
2. Initiative and Achievements in 2017
3. Multiple Sclerosis and Cystic Fibrosis Workshops: Aims, Objectives, Outcomes and Findings
4. Stakeholders actions
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EMA’s Patient Registry Initiative - Background

- Launched, September 2015
- Aims to strengthen contribution of patient registries to the benefit-risk evaluation of medicines
- **Pilot phase, 2016**: Stakeholder feedback encouraged an active role of EU regulatory network in supporting collaboration on the establishment and maintenance of disease registries
- **EMA study** - Bouvy et al. Pharmacoepidemiol Drug Saf. 2017: 65% of registries requested by CHMP are product registries
  Registries may support pharmacovigilance activities but have limitations hindering the creation of reliable, useful datasets
- 28th October 2016 - Patient Registries workshop

Workshop Report with recommendations
Registry: An organised system that uses observational methods to collect uniform data on a population defined by a particular disease, condition, or exposure, and that is followed over time

‘Broken Triangle’ barrier to better use of patient (disease) registries

Present...‘the broken triangle’

Future...MORE COOPERATION

Source: Nicola Ruperto, PRINTO
Patient Registries Initiative

- Led by a Cross-Committee Task Force of Scientific Committee members, National Competent Authority experts and EMA staff.

- Reports to the EMA’s Scientific Committees, Scientific Advice and Scientific Committees Board.
Patient Registries Initiative: Achievements in 2017

Communication and interaction with Stakeholders: Registry holders, PRAC, CHMP, PDCO, SA, Rapporteurs, Committee members, MAHs, MAAs, patients, funders, HTAs, NICE, EUnetHTA, FDA

MAH = Marketing Authorisation Holder; MAA = Marketing Authorisation Applicant

http://www.encepp.eu/
Workshops on **Cystic Fibrosis** and **Multiple-Sclerosis**

- **Cystic Fibrosis Workshop**: 14\(^{th}\) June
- **Multiple-Sclerosis Workshop**: 7\(^{th}\) July

**Why were these diseases chosen?**

- Multiple products marketed
- New products in the business pipeline
- Registries requested support for harmonisation
Workshop Aim: Outline agreement

- Common data elements
- Informed consents
- Governance
- Data protection
- Common protocols
- Registry interoperability
- Quality assurance

Final Outcomes → draft guidance for consultation → publication
Cystic Fibrosis & Multiple Sclerosis may act as models for other disease areas
**Workshop findings**

### Cystic Fibrosis Registries

- Mature collaborative registries landscape
  - Regional → national → single European registry
- Common registry platform
- Core common data elements collected systematically

### Multiple Sclerosis Registries

- Heterogeneous landscape
  - Two main registry holder groups
  - Post-Workshop, alliance discussion has commenced
- No single registry platform
- Limited collection of common data elements across registries

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**Both Registry Groups**

Keen to optimise use of data to support regulatory evaluations
Main actions for stakeholders 1

Cystic Fibrosis and Multiple Sclerosis Registries

✓ Confirmation on data sharing/access levels
✓ Processes for data requests and provision
✓ Systematic quality assurance measures
  • Data elements
  • Registry processes

Multiple Sclerosis Registries

✓ Agreement on core common data set
✓ Collaboration between main registry groups

Close the gap

- Regulatory authorities
- Pharmaceutical companies
- Registry holders/academia
Main actions for stakeholders 2

**MAHs / MAAs and Regulators**

- Consider use / availability of registry data early in the authorisation process and plan for its access and use where possible and/or appropriate.

- Current ‘reactive’ process → lead time loss
  - Consideration of registry data or information is mostly in response to Pharmacovigilance Risk Assessment Committee (PRAC) queries.
  - Little time for registries to adapt data collection / respond to needs.

- Adopt a pro-active process for registry consideration across the entire product lifecycle.
Main actions for stakeholders 3

Regulators

• Facilitate establishment of robust measures to confirm the quality of registry data
  ✓ Quality certification of registries may help provide assurance about data quality
  ✓ EMA Scientific Advice Working Party is exploring a qualification procedure with a European registry group

• Improve communications between registry holders, regulators and MAHs / MAAs

• Integrate registry consideration in regulatory processes from pre-submission through to post-authorisation follow up

• Align with other groups also active in the registries / real world data arena, e.g.
  ✓ Health technology assessment (HTA) groups
  ✓ European Commission initiatives
  ✓ Other regulators
Following the workshops

Increasing registry queries from Committees (e.g. PRAC) to EMA: eg. orthopaedics, inflammatory disorders, infectious diseases, haematology–oncology, including CAR-T cell therapies

Next steps

Communication and interaction with Stakeholders: Registry holders, PRAC, CHMP, PDCO, SA, Rapporteurs, Committee members, MAHs, patients, funders, HTAs, NICE, EUnetHTA, FDA
Next steps for Cross-Committee Task Force

- Facilitate CF and MS stakeholders in delivering agreed workshop actions
- Draft and publish key principles (from a regulatory perspective) on the use of registries in supporting medicines benefit-risk evaluations
- Establish methods for addressing EMA Committees’ requests about availability of and access to registry data that would support their decision-making
- Explore with EMA Committees on how systematic consideration of the inclusion of relevant registry data might be integrated early into their processes
- Continue inventory of registries in ENCePP Database
Embed registries pro-actively throughout regulatory processes

Committees
- PDCO
- CHMP
- CAT
- COMP
- PRAC

Regulatory Affairs support

PRIME
Scientific Advice

Validation Meeting

Business Pipeline

Pre-Submission Meeting

Evaluation Product Lead
Risk Management Specialist
Procedure Manager

Pre-Authorisation Evaluation

Post-Authorisation Evaluation
Pharmacovigilance

Proactive
Patient Registries Initiative

Embed registries throughout regulatory processes

Reactive

PRIME: Paediatric Committee; CHMP: Committee for Medicinal Products for Human Use; CAT: Committee for Advanced Therapies; COMP: Committee for Orphan Medicinal Products; PRAC: Pharmacovigilance Risk Assessment Committee;
Conclusions

✓ Paradigm shift from “MAH-owned product registry” to “joint collaboration with disease registry for long-term patient follow-up”

✓ Earlier discussions needed with registry holders during the authorisation process

✓ Gaps exist between the amount/type of data available in disease registries and data requested by regulators from MAHs
  ❖ Direct interactions between regulators and registry holders may help bridge the gaps

✓ Workshops reveal high interest from MAAs/MAHs and registry holders to engage
  ❖ Regulator encouragement is needed to ‘activate’ engagement

✓ Quality certification is likely to provide confidence in registry data
Setting-up the EMA inventory of registries in ENCePP

**Registry:** An organised system that uses observational methods to collect uniform data on a population defined by a particular disease, condition, or exposure, and that is followed over time.

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<td>Disease/Patient registries</td>
<td>Product registries</td>
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<td>European registries</td>
<td>Non-European registries</td>
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<td>Special attention to rare diseases</td>
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<td>Multinational, National and regional registries</td>
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Registries by Therapeutic areas

N = 47 registries
by 15th September 2017
Next steps & Conclusions

- The EMA started the inventory:
  - Based on our own searches and registries we knew about them
- Routine work at the PV department
  - The EMA approaches registry holders
  - Patient registries are invited to join the ENCePP resources database and add their registry details.
- Inventory aimed to facilitate interaction between stakeholders.
- Guidance on how to upload and search for patient registries to be published soon.
  - Registries data entry harmonization ("data source" classification, ...)


EMA registry initiative

Scientific Lead: Patricia McGettigan
Initiative coordinator: Mireia Castillon
Scientific support and inventory of registries: Carla Alonso Olmo
Administrative support: Valerie Muldoon
Thank you for your attention

Further information

Contact us at EMAregistries@ema.europa.eu

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

Telephone  +44 (0)20 3660 6000  Facsimile  +44 (0)20 3660 5555
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