EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Patient Registries Initiative
Background, Achievements, Next steps

21 November 2017
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:

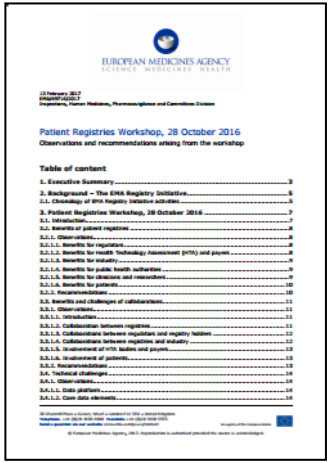
   Aims, Objectives, Outcomes and Findings
4. Stakeholders actions
5. Next steps
6. Conclusion

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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Patient Registries Workshop, 28 October 2016
Observations and recommendations arising from the workshop

Table of content

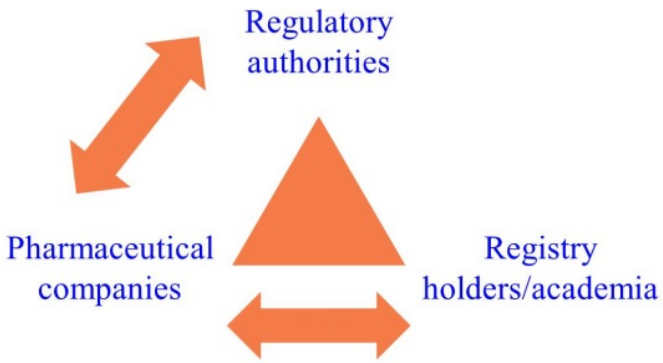
- **28th October 2016 - Patient Registries workshop**

Workshop Report with recommendations

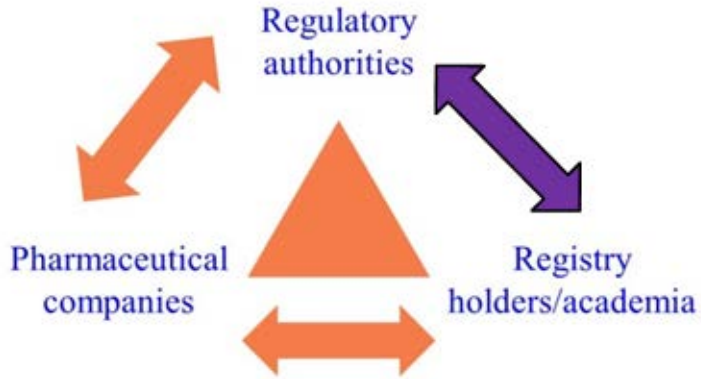
Registry: An organised system that uses observational methods to collect uniform data on a population defined by a particular disease, condition, or exposure, and that is followed over time

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

Present...'the broken triangle'



Future...MORE COOPERATION



Source: Nicola Ruperto, PRINTO

Patient Registries Initiative

TO FACILITATE

Harmonisation of data collected in Disease Registries

TO PROTECT

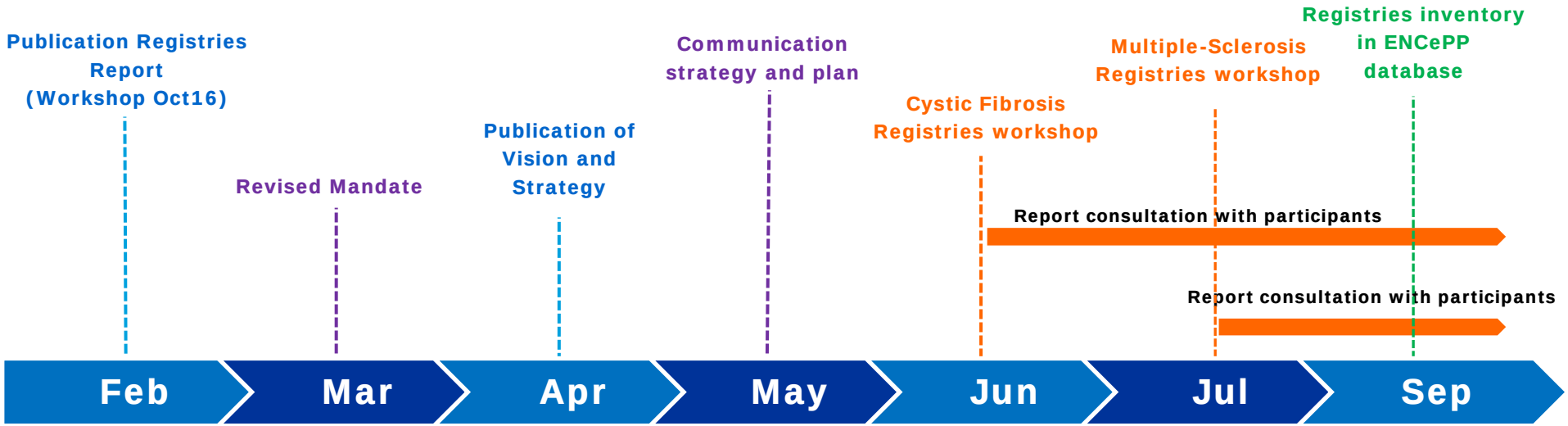
public health through better use of registry data to support benefit risk evaluation

TO CAPITALISE

On networks of Registry Stakeholders

- 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: 14th June

Multiple-Sclerosis Workshop: 7th 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

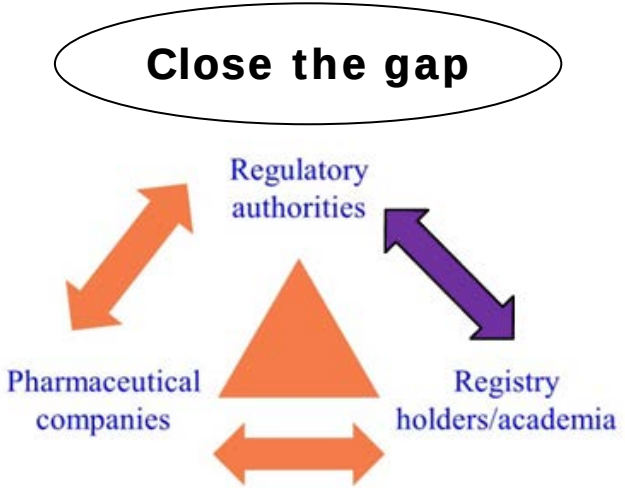
Limited collection of common data elements across registries

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

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

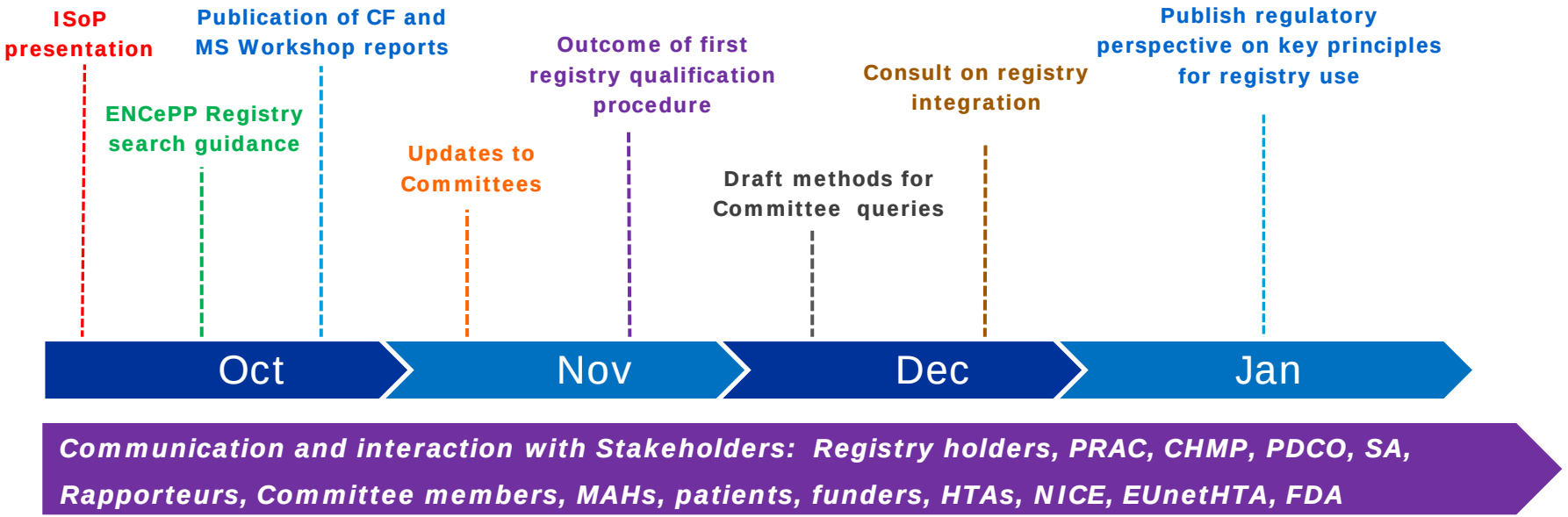
Regulators

- 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



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

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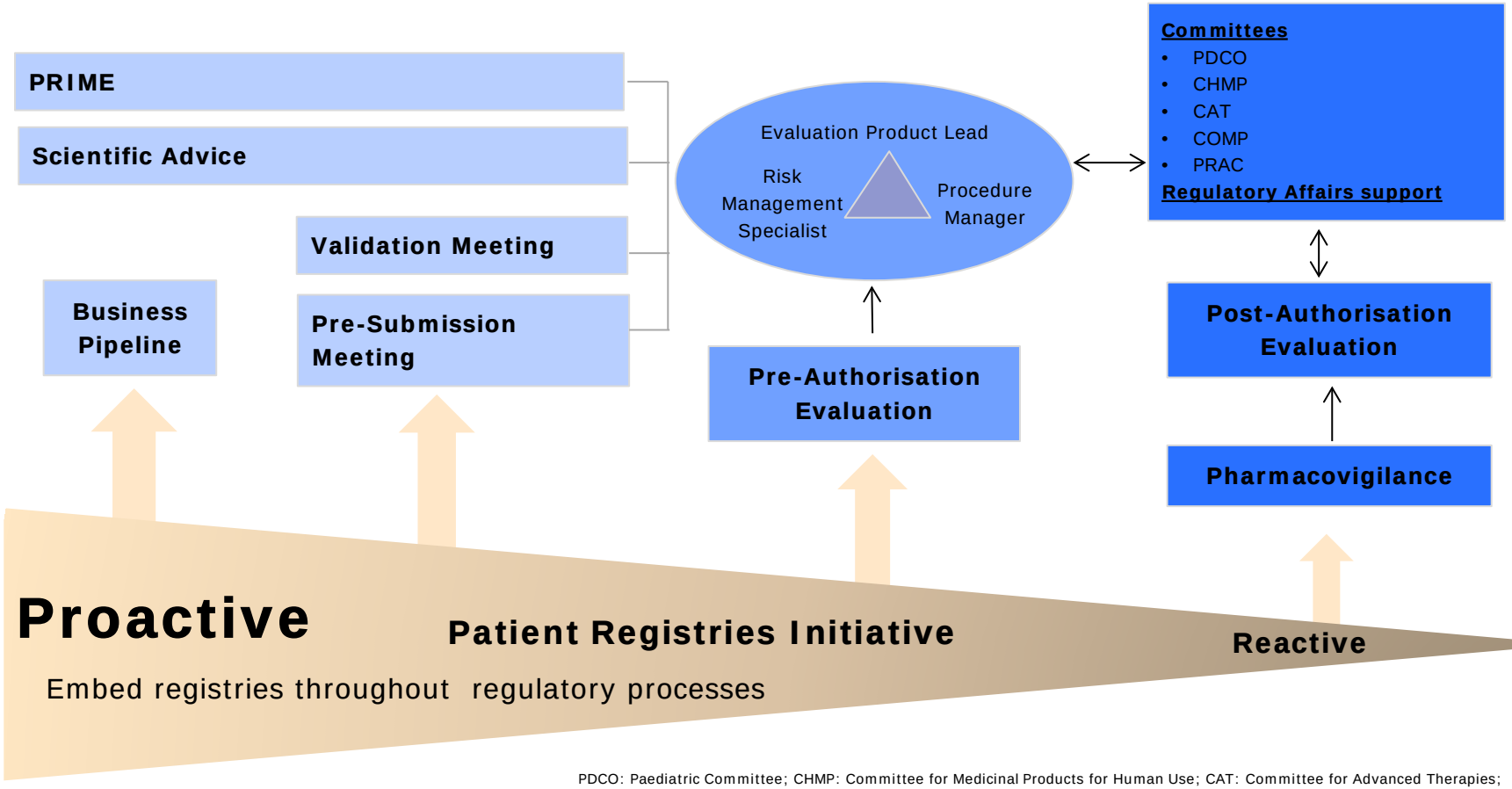


PDCO: Paediatric Committee; CHMP: Committee for Medicinal Products for Human Use; CAT: Committee for Advanced Therapies; COMP: Committee for Orphan Medicinal Products; PRAC: Pharmacovigilance Risk Assessment Committee;

Conclusions

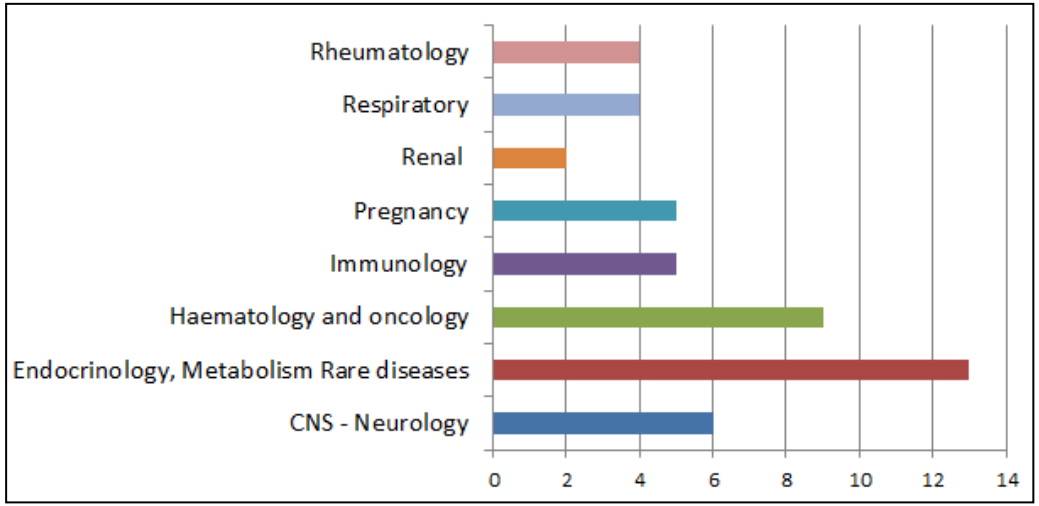
- ✓ Paradigm shift from “MAH-owned product registry” to “joint collaboration with disease registry for long-term patient follow-up”
- ✓ Earlier discussions needed with registry holders during the authorisation process
- ✓ Gaps exist between the amount/type of data available in disease registries and 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.

Included	Excluded
Disease/Patient registries	Product registries
European registries	Non-European registries
Special attention to rare diseases	
Multinational, National and regional registries	

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

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