



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Patient Registries Initiative

Background, Achievements, Next steps

21 November 2017
ENCePP Plenary meeting





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

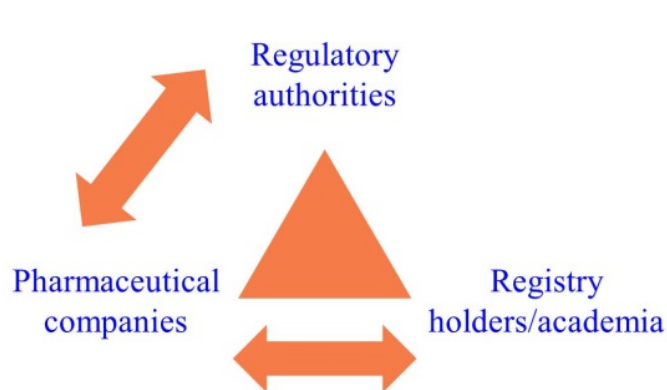


[Workshop Report with recommendations](#)

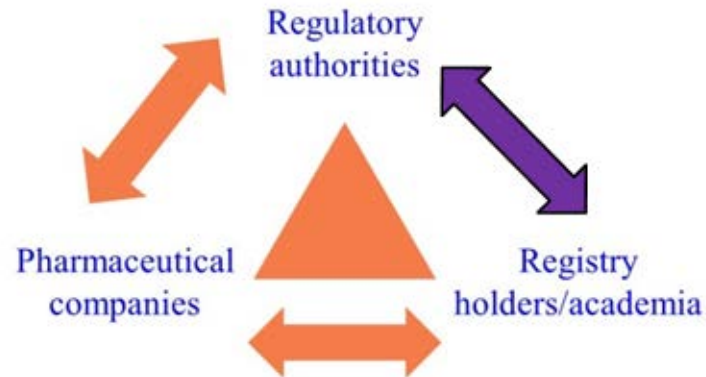
Registry: An organised system that uses observational methods to collect uniform data on a population defined by a particular disease, condition, or exposure, and that is followed over time

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

Present...'the broken triangle'



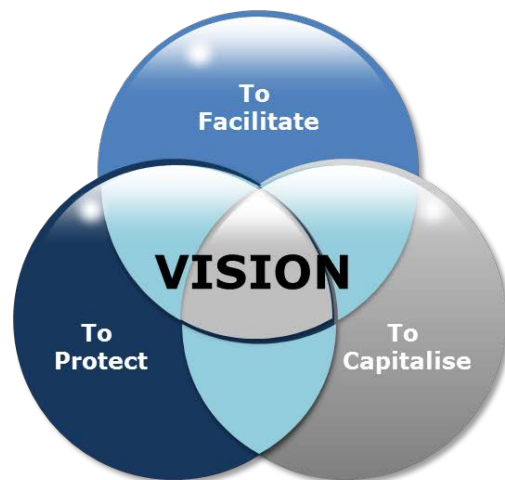
Future...MORE COOPERATION





Patient Registries Initiative

TO FACILITATE
Harmonisation of data
collected in Disease Registries



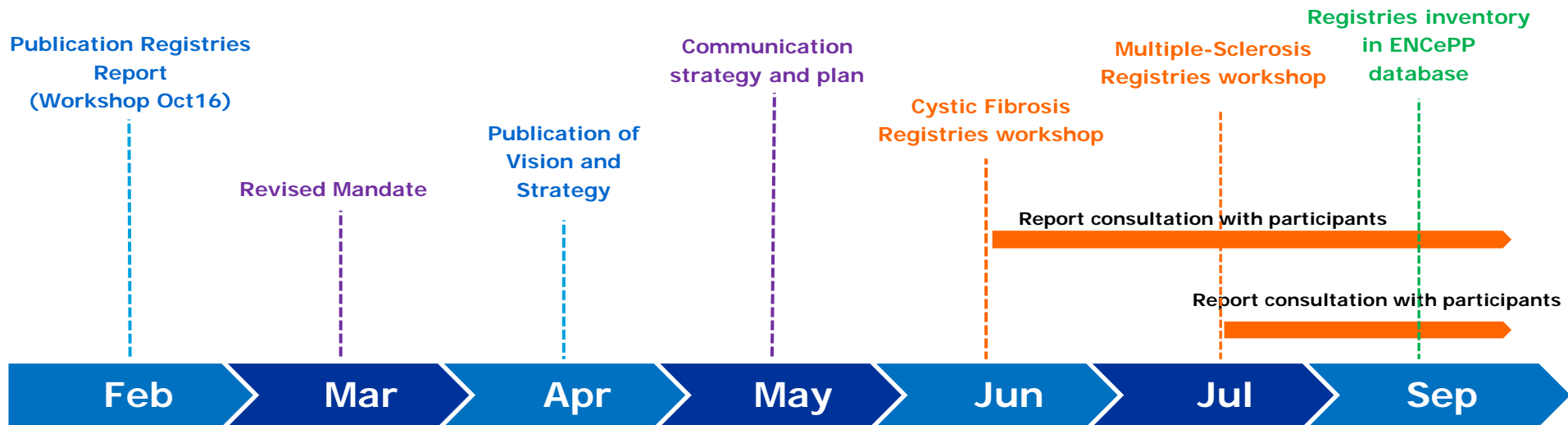
TO PROTECT
public health through
better use of registry data
to support benefit risk
evaluation

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



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

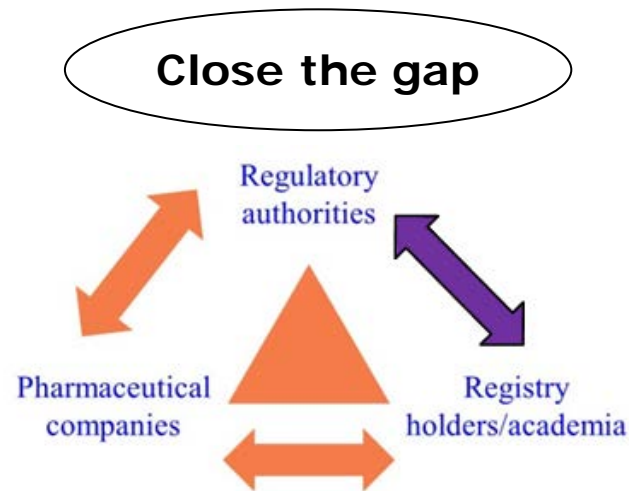
Main actions for stakeholders 1

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

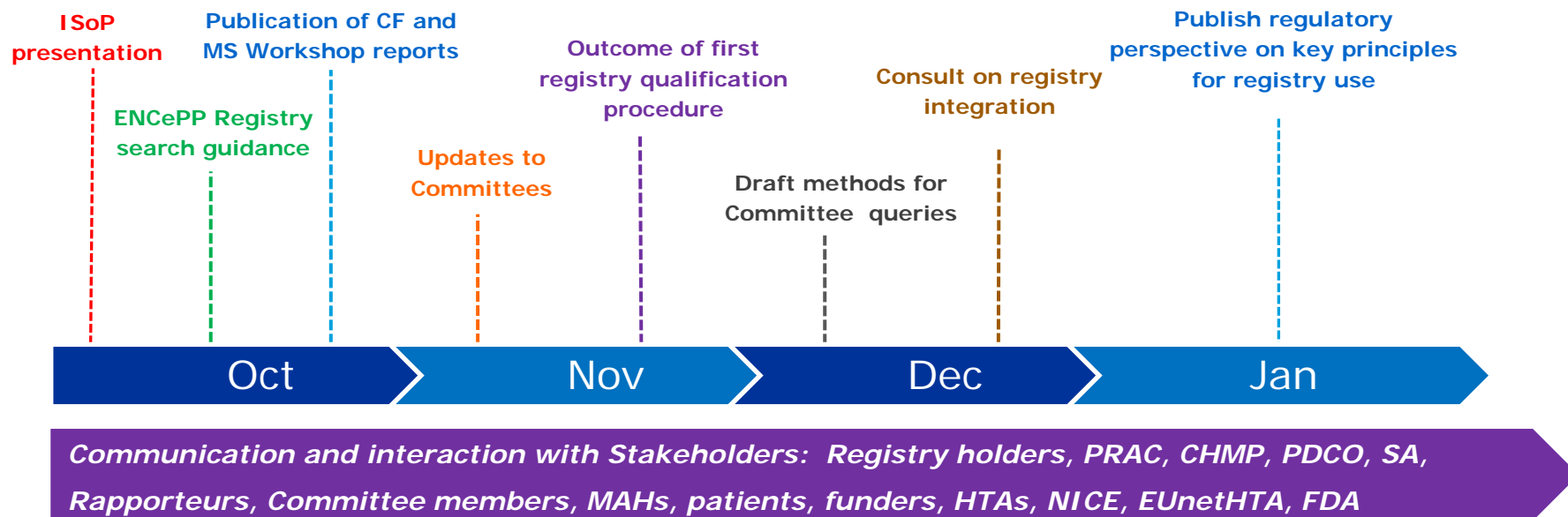
- Facilitate establishment of robust measures to confirm the quality of registry data
 - ✓ Quality certification of registries may help provide assurance about data quality
 - ✓ EMA Scientific Advice Working Party is exploring a qualification procedure with a European registry group
- Improve communications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

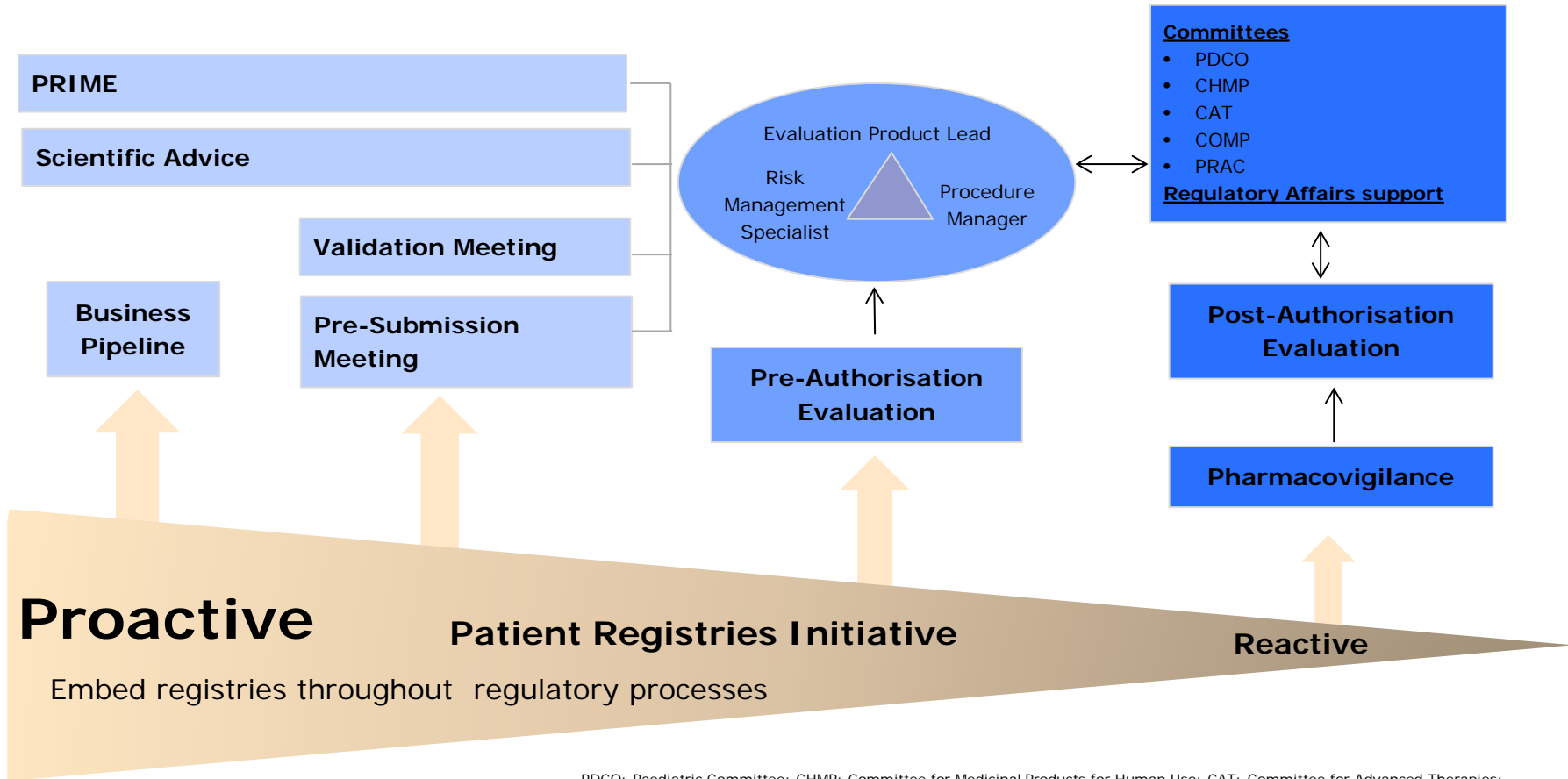




Next steps for Cross-Committee Task Force

- ✓ 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



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



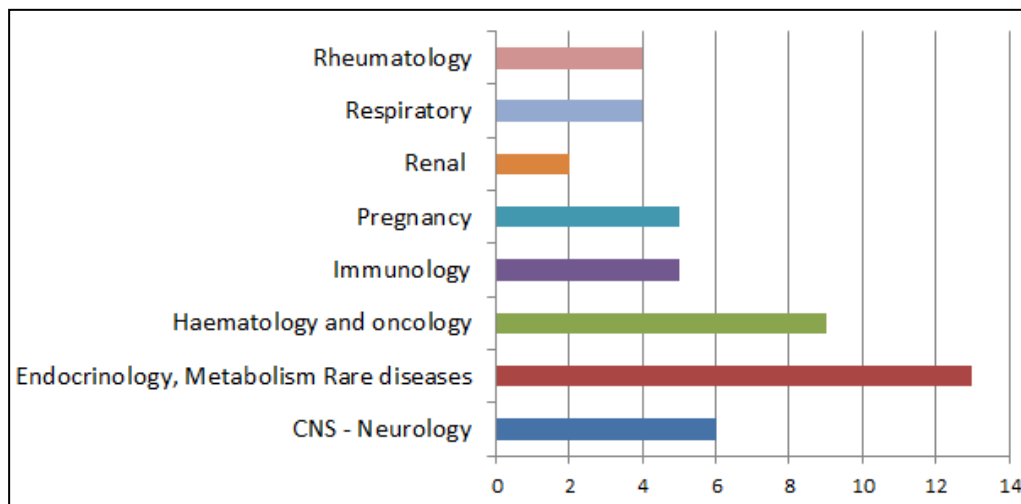
Setting-up the EMA inventory of registries in ENCePP

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

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

Send a question via our website www.ema.europa.eu/contact

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