



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Patient Registries Initiative

Lessons learned from the recent workshops

21 September 2017

11th Stakeholders forum on the pharmacovigilance legislation meeting



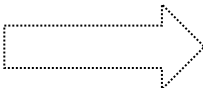


Agenda

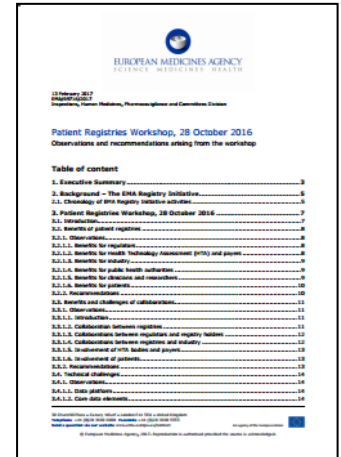
- ✓ Background
- ✓ Governance structure and responsibilities
- ✓ Vision of the EMA's Patient Registries Initiative
- ✓ Achievements in 2017
- ✓ Workshops on Cystic Fibrosis and Multiple-Sclerosis
- ✓ Next steps
- ✓ Conclusions



EMA's Patient Registry Initiative - Background

- Launched in September 2015
- Aims to explore ways of expanding the use of patient registries by introducing and supporting a more systematic and standardised approach to their contribution to the benefit-risk evaluation of medicines within the European Economic Area
- Stakeholder feedback encourages an active role of EU network in supporting collaboration on the establishment and maintenance of disease registries
- **28th October 2016 - Patient Registries workshop** 

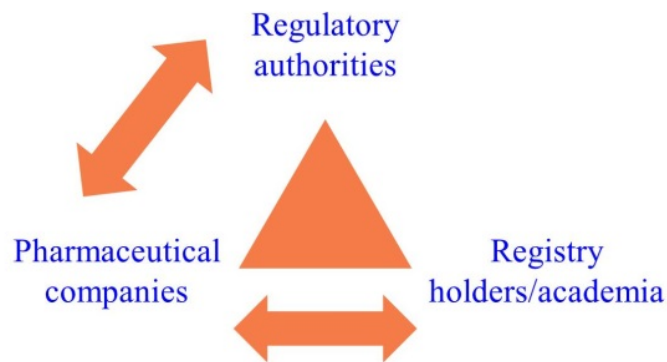
http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000658.jsp



[Workshop Report with recommendations](#)

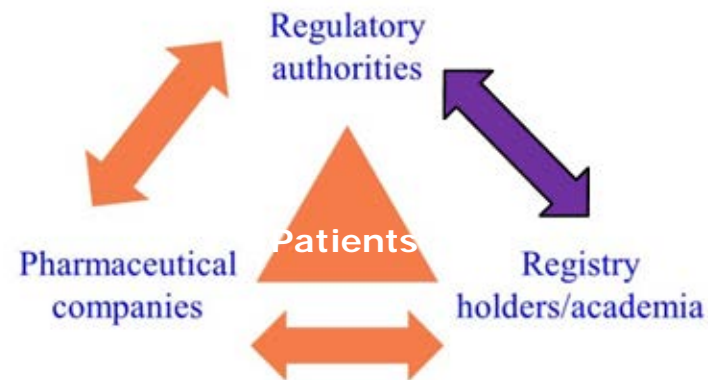
Facilitation of **interactions** between regulators and registry holders

Present... 'the broken triangle'



Source: Nicola Ruperto, PRINTO

Future... MORE COOPERATION





Governance structure and responsibilities

- The Initiative is led by a **Cross-Committee Task Force**.
- Composed of: **Scientific Committee** members, experts from **NCA**s and **EMA staff**.
- Co-chaired by a NCA representative elected by the Task Force and an EMA representative elected by EMA.

The Task Force oversees the establishment and running of the Patient Registry Initiative. It reports to the EMA's scientific committees, Scientific Advice and to the Scientific Committees Board.



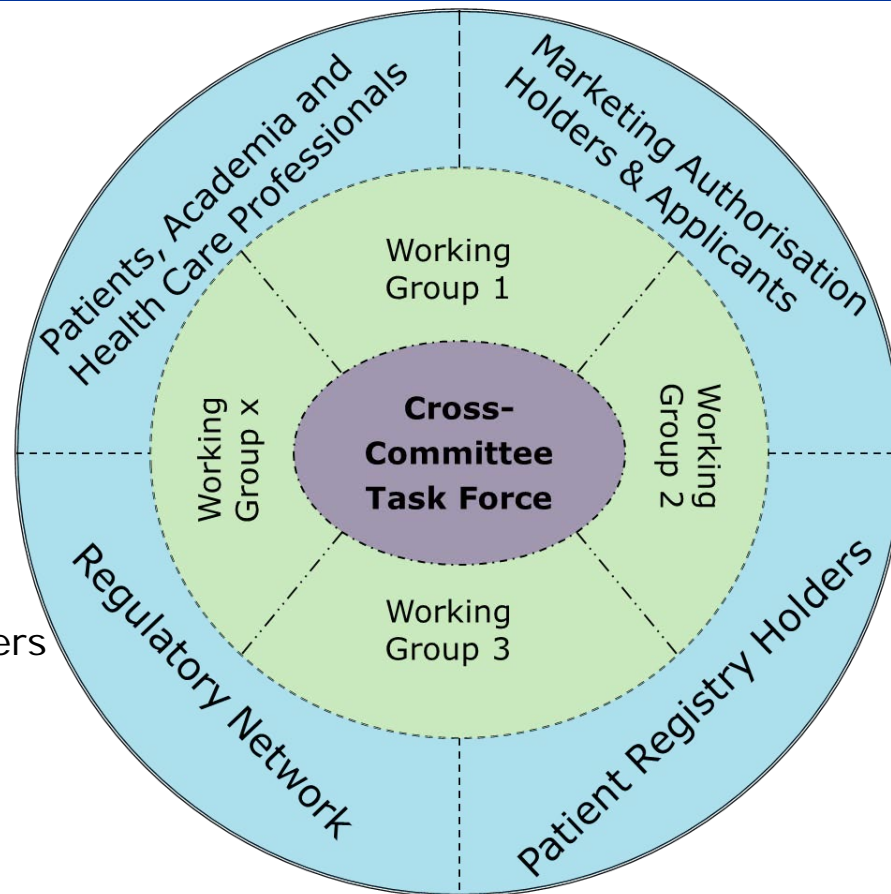
Governance

Mandate and Strategy

Revised & published on EMA website

Working Groups

- Working groups are established on direction of the Task Force
- Undertake specific disease-area tasks, liaising with the appropriate stakeholders (Registry holders, patients, MAHs etc)
- Disband when task completed





Vision of the 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

Patient Registries Initiative



Achievements in 2017

Publication Registries

Report
(workshop Oct16)

Revision Mandate
Patient Registries

Publication new
Vision and
Strategy for
Patient Registries

Communication
strategy and
communication
plan approved

Cystic Fibrosis
registries
workshop

Multiple-Sclerosis
Registries workshop

Report consultation with participants

Report consultation with
participants



Communication and interaction with Registries stakeholders : NICE, EUNethA, FDA, Registry holders, PRAC, CHMP, Rapporteurs, Committee members, MAH, payers, HTAs, patients



Workshops on *Cystic Fibrosis and Multiple-Sclerosis*

Cystic Fibrosis Workshop: 14th June

Multiple-Sclerosis Workshop: 7th July

Why these diseases were chosen?

- ✓ Number of products have been marketed
- ✓ New products on the business pipeline
- ✓ EU Disease Registries have 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 methods

- ✓ Three working groups:
 - 1) Common data elements
 - 2) Consent and Governance
 - 3) Interoperability and Quality
- ✓ Pre-work assigned to working group members
 - ✓ Completed by almost all participants – Collated & notes circulated by EMA team
 - ✓ Participants arrived prepared for the discussions
- ✓ Dynamic and interactive workshop
 - ✓ Perspectives of stakeholders were captured
 - ✓ Opportunity to share critical issues of each interested party
 - ✓ Draft observations & recommendations reviewed by all participants
- ✓ Draft Reports currently in Review



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

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

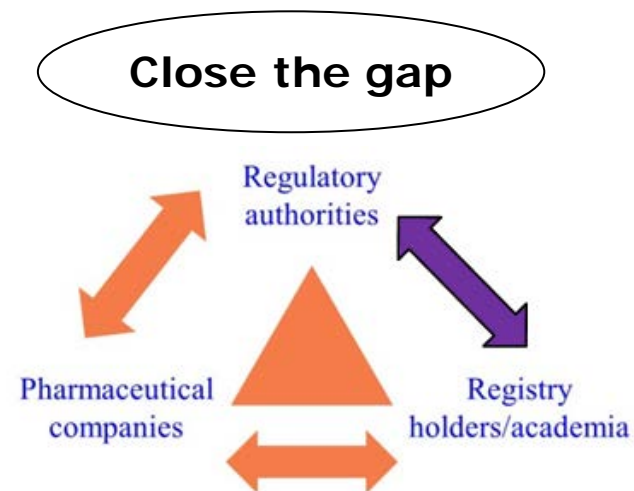
Main actions for stakeholders

Cystic Fibrosis & Multiple Sclerosis

- ✓ Confirmation on data sharing/access levels
- ✓ Processes for data requests and provision
- ✓ Systematic quality assurance measures

Multiple Sclerosis

- ✓ Agreement on core common data set





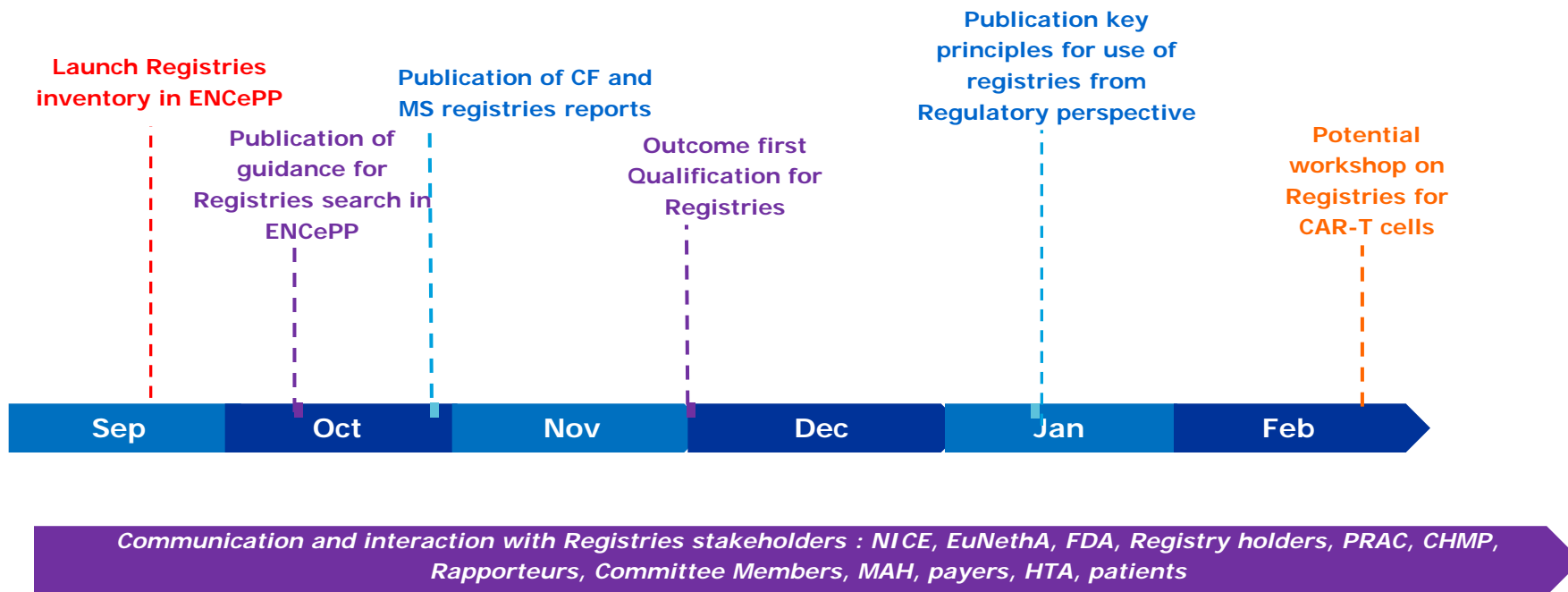
Agreements from the Workshops

Participants agreed

- ✓ To optimise the use of registry data → support **regulatory benefit-risk evaluations** where possible and / or appropriate
- ✓ To consider use / availability of registry data early in the authorisation process and plan for its access and use
- ✓ To establish robust measures to confirm the **quality of registry data**
 - independent quality certification may provide confidence
- ✓ To **improve communications** between registry holders, regulators and marketing authorisation holders / applicants



Next steps





Data Standards – quality certification

- ✓ The EMA is exploring the possibility for a qualification process for registries
- ✓ A first registry group is in discussions with EMA Scientific Advice

Creation of an Inventory of Registries

- ✓ Inventory of Registries is being hosted on ENCePP platform
- ✓ European Registry-holders invited to have their Registry information included in ENCePP resources database – positive uptake

<http://www.encepp.eu/>



European Network of Centres
for Pharmacoepidemiology and Pharmacovigilance





Conclusions

- ✓ Patient follow-up through disease registries is increasingly requested by regulators to industry, especially in the context of new treatments for rare diseases
- ✓ Shift of paradigm from “product registry owned by single company” to “(joint) collaboration with disease registry for long-term patient follow-up”
- ✓ Earlier discussions with registry holders during the development process are needed
- ✓ Gap between the amount and type of data collected in disease registries and data requested by regulators to industry – direct interactions between regulators and registry holders may help fill the gap
- ✓ Experience from CF and MS registries shows high level of interest from companies and registry holders to collaborate
- ✓ Independent quality certification may provide confidence in registry data
- ✓ EU regulatory network to develop tools to support use of data from disease registries



Thank you for your attention

Further information

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