Patient Registries Initiative
Pre-work package

July 2017, Multiple Sclerosis Workshop

- Background of the Workshop
- The EMA Patient Registries Initiative
- Workshop: Expected Outcomes of the day
- Landscape: products, patients, stakeholders
- Participant Pre-work - to be returned to EMA by **14th June**
- The EMA Patient Registries Initiative - Governance structure
- Useful links
- Next steps

For some products, regulators may require MAA/MAHs to undertake further risk-benefit or outcomes evaluations.

In some cases, registry data may be suitable. Product registries are not encouraged owing to their inherent limitations.
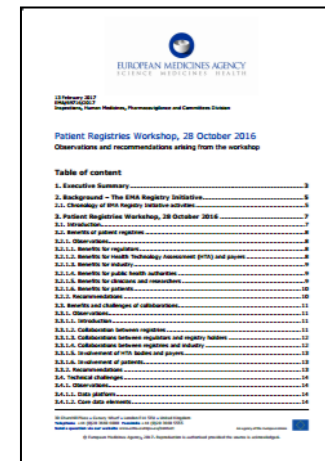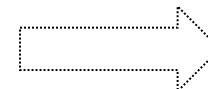
The EMA encourages the use of patient (or disease) registries and wishes to provide guidance to optimise their use.

EMA objectives:

1. **Coordination between ongoing initiatives at national and international levels**
2. **Harmonised protocols, scientific methods and data structures**
3. **Data sharing and transparency**
4. **Registry sustainability.**

Therefore, the required pre-work is key for meaningful participation in the workshop – this is to be submitted by each participant by 14th June

EMA Patient Registries Initiative

- 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

- Stakeholders [Registry holders, Patients, Industry, Reimbursement & HTA agencies] wish an active role of the EU network in supporting collaboration on use of disease registries

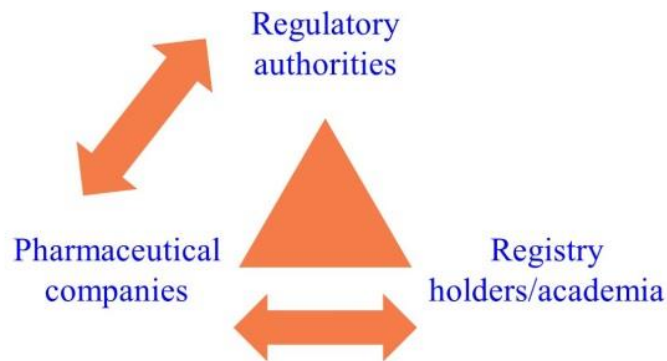- **28th October 2016 - Patient Registries workshop**

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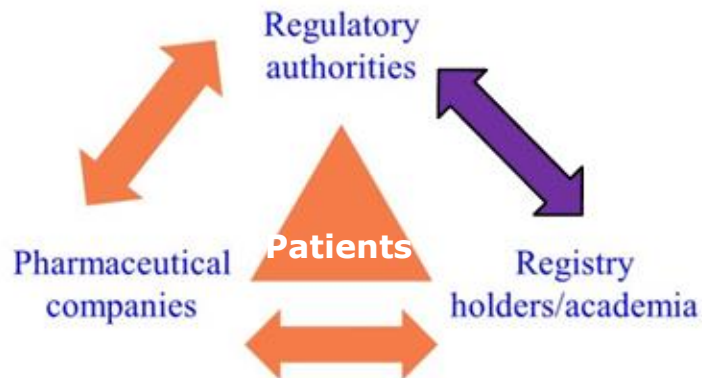

Workshop Report with recommendations

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

Present...'the broken triangle'

Future...MORE COOPERATION

Source: Nicola Ruperto, PRINTO

EU-Network – Stakeholder Recommendations

✓ **Common core data elements** for registries

✓ **Specific data elements** for treatment classes / innovative products

✓ **Safety aspects** (e.g. reporting of ADEs)

✓ **Data quality**

✓ **Governance** for interactions between stakeholders

- *data ownership, data analysis, data sharing, registry interoperability*

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

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Assigned pre-work must be completed to achieve outline agreement on:

1	Common data elements that are needed by all stakeholders irrespective of the clinical/ academic/ regulatory/ health technology assessment/ reimbursement purposes of registries
2	Informed consents and governance, data protection
3	Common protocols and registry interoperability, quality assurance measures to support regulatory medicines evaluation purposes

Final Outcomes → draft guidance for wider stakeholder consultation → publication → model for other disease area registries

Multiple Sclerosis landscape

- Estimates:>700,000 persons affected directly by multiple sclerosis (MS) in Europe

- Prevalence varies, being higher in northern European latitudes, lower in southern latitudes (Estimates are highly variable: range 20-40/10,000 in Northern Europe to 2-20/10,000 in Southern Europe)

- Costs of managing MS in Europe estimated at 15 billion euros per year.

- Currently there are 11 products available on the market and further products are in development or under evaluation at the EMA.

- **Stakeholders**

  - Patient Representatives

  - Patient Registry Holders

  - Regulators

  - Marketing Authorisation Holders

  - Reimbursement & HTA agencies

Common data elements

- Prepare a list or illustration of the data elements currently collected in your own registry.

- Propose a minimal core set of common data elements that you consider essential and that could feasibly be collected by registries in your disease area. Include patient reported outcomes.

- Describe your experience of adverse effect/ pharmacovigilance/ risk-benefit reporting in your registry

- List what you view as the information gaps in MS treatment studies, from a patient perspective, that you think registries might be able to fill.

- Propose how longitudinal information could be collected feasibly on investigations/results and on all drugs/treatments taken by patients.

Consents and Governance

- Propose what you consider would be appropriate data-sharing between registries and with other stakeholders including industry and regulators. Prepare to discuss the possibility for patient level data to be provided to regulators.

EUROPEAN MEDICINES AGENCY

Common Data Elements

- Prepare a list or illustration of the data elements currently collected in your own registry.

- Propose minimal a core set of common data elements that you consider essential and that could feasibly be collected by all registries.

- Describe your experience of pharmacovigilance/adverse effect/ risk-benefit reporting and frequency of reporting requested by industry for regulator-requested post-marketing studies, if applicable.

- List what you view as the limitations in MS management and treatment studies that you think registries might be able to address.

- Propose how longitudinal information could be collected in your registry on investigations/results and on all drugs/treatments taken by patients. *Include data collection protocols that you have used and that worked well in the studies concerned.*

Data Quality and Governance

- Provide a list of indicators that you think could feasibly be measured to verify data and to assure quality.

- Propose how your suggested verification measures could be operationalised by Registry holders.

- Propose what you consider would be appropriate governance arrangements to permit data sharing between registries and with other stakeholders including industry and regulators.

- Prepare to discuss /clarify the possibility of patient level data provision to regulators and frequency of submission of such data.

Common data elements

- List the limitations and gaps you have identified in working with registry data.

- Propose a minimal core set of common data elements that that you consider essential and that you think might feasibly be collected by all registries.

- Describe your experience of registry data for meeting pharmacovigilance requirements, including level of detail and frequency.

- List what you view as the information gaps in MS treatment studies that you think registries might be able to fill.

- Describe the types of longitudinal information that you consider MS registries should collect in order to be of assistance for regulators.

Pre-work to be done by Regulators 2

Data Quality and Governance

- Draft a list of indicators that you think could feasibly be sought to verify their data and assure quality.

- Propose how your suggested verification measures could be operationalised by Registry holders.

- List the data quality standards that need to be addressed in order to consider data fit for regulatory post-authorisation / non-interventional studies taking Good Vigilance Practices into account.

- Propose what you consider would be appropriate governance arrangements to permit data sharing between registries and with other stakeholders including industry and regulators.

- Prepare to discuss the possibility of patient level data provision to regulators and frequency of submission of such data.

Common data elements

- List the information / data gaps you have identified in working with registries.

- Propose a minimal core set of common data elements that you consider essential and that you think might feasibly be collected by all registries.

- Describe your experience of registry data for meeting pharmacovigilance requirements, including level of detail and frequency of collection.

- List what you view as the information gaps in MS treatment studies that you think registries might be able to fill.

- Describe the types of longitudinal information that you consider MS registries should collect in order to be of value for MAHs.

Data Quality and Governance

- What do you consider are the enablers and barriers to the use of existing disease registries for post-authorisation studies? This should include consideration of data sharing.

- List the data quality standards that need to be addressed in order to consider data fit for regulatory post-authorisation / non-interventional studies taking Good Vigilance Practices into account.

- Draft a list of indicators that you think could feasibly be measured to verify data and assure quality

- Propose how your suggested verification measures could be operationalised by Registry holders.

- Propose what you consider would be appropriate governance arrangements to permit data sharing between registries and with other stakeholders including industry and regulators.

Common Data Elements and Data Quality

- List the data gaps you have identified in working with registries.

- Propose a minimal core set of common data elements that you consider essential and that you think might feasibly be collected by all registries.

- List what you view as the information gaps in MS treatment studies, from reimbursement / HTA perspectives, that you think registries might be able to fill.

- What longitudinal outcomes information and data are of interest to reimbursement / HTA agencies? Propose how this might be collected by registries.

- What are appropriate data standards from reimbursement / HTA perspectives?

- What source data verification is necessary from reimbursement / HTA perspectives?

Consents and Governance

- Propose what you consider would be appropriate levels of data sharing between registries and reimbursement / HTA agencies.

- Prepare to discuss /clarify any need for patient level data provision to payers, and frequency of submission.

Governance structure and responsibilities

- The Initiative is led by a **Cross-Committee Task Force**

- Composed of: **Scientific Committee** members, experts from **NCAs** 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.
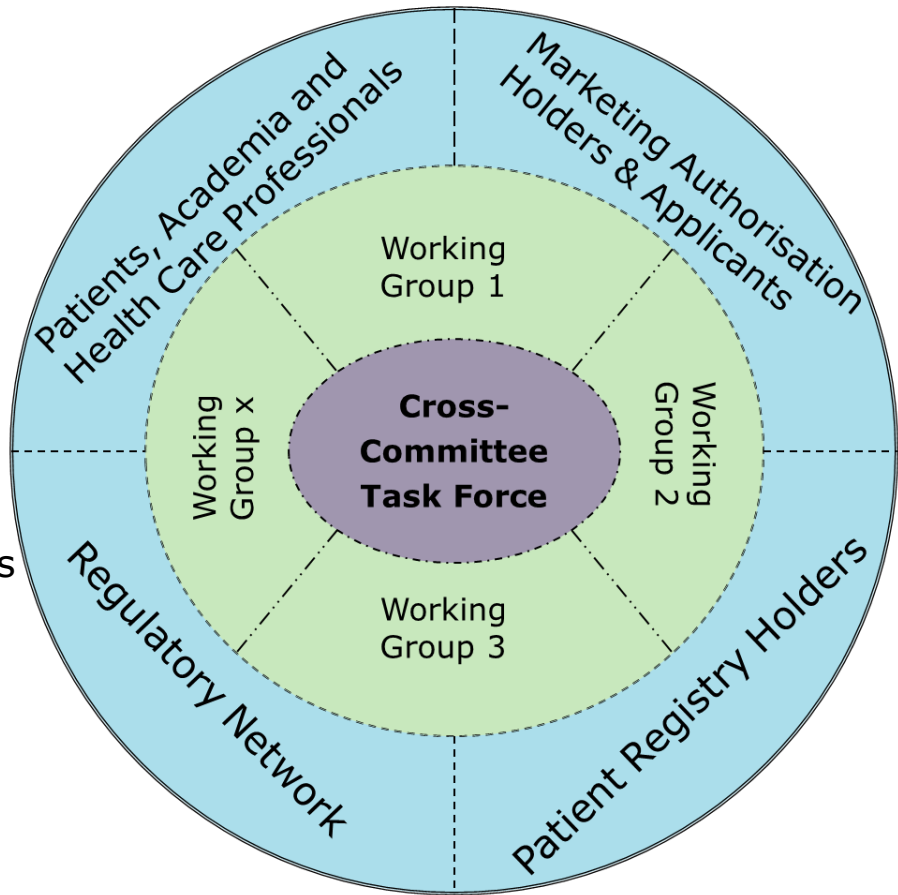
Governance

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

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

Useful links

- EMA Website – Patient Registries Initiative Subpage

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

- Workshop Report - Recommendations

http://www.ema.europa.eu/docs/en_GB/document_library/Report/2017/02/WC500221618.pdf

Next steps

- Pre-work to be submitted by 14th June 2017

- Should you have additional suggestions, please share them

- Should you have any question, please contact us at:

    EMAregistries@ema.europa.eu

Thank you for your attention

Further information

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