


 EUROPEAN MEDICINES AGENCY
 SCIENCE MEDICINES HEALTH

Patient Registries Initiative Pre-work package

June 2017, Cystic Fibrosis Workshop

EMA Patient Registries Cross-Committee Task Force

Pre-work package content

- 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 **2nd June**
- The EMA Patient Registries Initiative - Governance structure
- Useful links
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Background of the workshop

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 2nd 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**

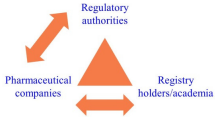
http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000658.jsp

[Workshop Report with recommendations](#)

The EMA Patient Registries Initiative

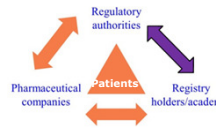
Facilitation of **interactions** between regulators and registry holders

Present... 'the broken triangle'



Source: World Report, 2007

Future... MORE COOPERATION



The EMA Patient Registries Initiative

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

Workshop: Expected outcomes of the day

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

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Cystic Fibrosis landscape

- Cystic fibrosis (CF) affects approximately 0.7 in 10,000 people in the European Union (EU), equivalent to a total of around 36,000 people, and below the ceiling for orphan designation, 5 people in 10,000.
- Currently there are 9 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

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Pre-work to be done by Patient Representative

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 CF 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.

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Pre-work to be done by Patient Registry Holders 1

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 CF 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*

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Pre-work to be done by Patient Registry Holders 2

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.

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Pre-work to be done by Regulators 1

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 CF treatment studies that you think registries might be able to fill.
- Describe the types of longitudinal information that you consider CF registries should collect in order to be of assistance for regulators.

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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.

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Pre-work to be done by Marketing Authorisation Holders 1

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 CF treatment studies that you think registries might be able to fill.
- Describe the types of longitudinal information that you consider CF registries should collect in order to be of value for MAHs.

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Pre-work to be done by Marketing Authorisation Holders 2

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.

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Pre-work to be done by Reimbursement & HTA agencies 1

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 CF 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?

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Pre-work to be done by Reimbursement & HTA agencies 2

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 reimbursement / HTA agencies, and frequency of submission.

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The EMA Patient Registries Initiative

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.

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The EMA Patient Registries Initiative

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

The EMA Patient Registries Initiative

Vision

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

VISION

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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

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Next steps

- Pre-work to be submitted by 2nd June 2017
- Should you have additional suggestions, please share them
- Should you have any question, please contact us at: EMAregistries@ema.europa.eu

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Thank you for your attention

Further information

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