

Lessons from the EMA Patient Registries Initiative

STAMP Commission Expert Group 8th June 2018

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EMA Relocation: Business Continuity

Due to uncertainties on staff loss and other relocation implications, **all activities** on real world data, big data and registries between September 2018 and June 2020, will need to be prioritised in the context of **business continuity planning**



In this presentation:

- Core concepts
- > EMA-imposed registries experiences and case for the Registries Initiative
- The EMA Patient Registries Initiative Strategy
- EMA Registry workshops lessons learned?
- Parallel Regulatory HTA engagement
- How can regulators support use of disease registries?
- Conclusions
- 2

What are the core concepts?



Registry

An organised system that uses observational methods to collect uniform data on specified outcomes in a population defined by a particular disease, condition or exposure.

Regulators generally prefer *patient (disease) registries* over *product registries*They gather insights on clinical outcomes of conditions with different

treatments, rather than on the outcomes of specific treatments

- •They allow comparisons
- •They are generally better integrated into health care systems.

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Background: EMA imposed registries

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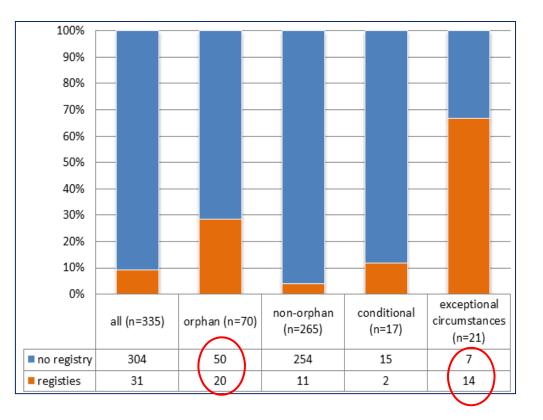
Registries may be requested of / or imposed on companies as part of risk management plans including for:

- advanced therapies
- orphan products
- medicinal products paediatric use

Examination of registries imposed as an obligation at the time of authorisation for centrally-authorised products, 2005-2013

Overall, use of a registry imposed for 10% of products authorised

Bouvy et al. PDS 2017;26(12):1442-50 (EMA study)



Background: Problems observed with imposed registry studies



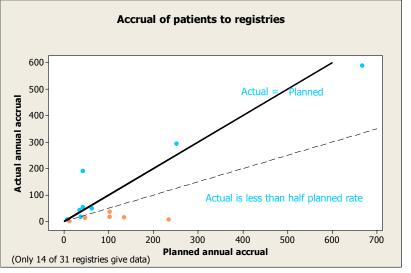
Registry issues	N (24 Total)	%
No problem reported	9	37.5
Low accrual rate	13	54.2
Delayed start	9	37.5
Protocol amendment required	9	37.5
Low quality / missing data	3	12.5
Low use of product	3	12.5
Low enrolment for other reasons	3	12.5

16/24 (66%) registries were product specific19/24 (80%) were new registries7 registries never commenced

Actual vs. planned number of patients included

< 50% inclusion

Analysis of European Public Assessment Reports, study protocols, PSUR and PSUR assessment reports. Data lock: 30 June 2015Bouvy et al. PDS 2017;26(12):1442-50 (EMA study)PSUR = Periodic Safety Update Report



Reasons for problems encountered



Approach to registries often suboptimal in scientific and resource terms:

- \succ Existing registries not fully exploited \rightarrow duplication of efforts and inefficiencies
- > Discrepancy between data collected by registries and data requested by regulators
- Existing patient (disease) registries were not set up for regulatory purposes
- Challenges in using registries for regulatory studies:
 - Recruitment: lack of physician engagement due to administrative burdens, patient consent, low product usage and competing registries
 - > **Data quality**: representativeness of registry population, missing data
 - > Lack of consistent data **quality control**
 - Sustainability (funding)
- So companies may prefer to establish individual product registries

The EMA's Patient Registry Initiative



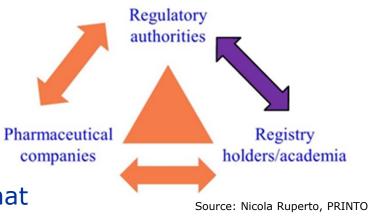
- Launched, September 2015 Cross-Committee Task Force
- Aims to facilitate use of patient (disease) registries by introducing and supporting a systematic approach to their contribution to the benefit-risk evaluation of medicines
- Pilot phase, 2016: Stakeholder feedback encouraged an active role of EU network in supporting collaboration for greater utilisation of disease registries
- 28th October 2016 Stakeholder workshop: focus on methods
- Specific workshops
 - June 2017: Cystic fibrosis registries
 - July 2017: Multiple sclerosis registries
 - February 2018: Registries for CAR T-cell therapies
 - June 2018: Haemophilia (Factor VIII) registries

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Strategy



- To promote dialogue between regulators, companies and registry holders to understand barriers and opportunities of using disease registries.
 - To clarify concepts: registry vs. study that may be registry-based



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EMA registries workshops



Cystic Fibrosis Registries Workshop: 14th June 2017

Multiple-Sclerosis Registries Workshop: 7th July 2017

CAR T-Cell therapies Registries Workshop: 9th February 2018

Participants: regulators, companies, registry holders, health technology assessment bodies, patient and health care representatives

Diseases selection?

- Products recently authorised or authorisation process ongoing
- New products business pipeline
- EU disease registries have requested support for harmonisation
- On-going qualification procedures for two EU-wide registry platforms

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EMA registries workshops



Cystic Fibrosis Registries

Well-organised Europe-wide network; Core common data elements in place

Multiple-Sclerosis Registries

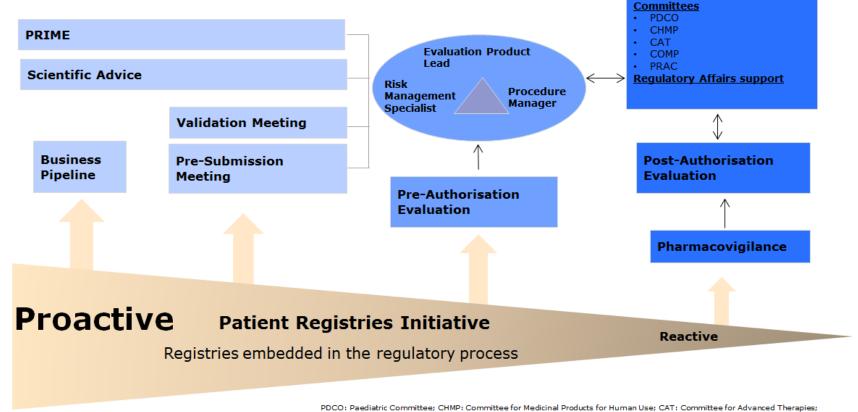
Two registry groupings: European MS Platform (patients) & Big MS Data (academic); Limited between-grouping collaboration; No within-group agreement on core common data elements

CAR T-Cell therapies Registries

European and US registry networks; Collaborative ; Data element harmonisation ongoing

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Proactive early discussion during the regulatory process



COMP: Committee for Orphan Medicinal Products; PRAC: Pharmacovigilance Risk Assessment Committee;

1:



Governance

- Regulators and marketing authorisation holders / applicants (MAHs/MAAs)
 - Need to be aware of data that can <u>feasibly</u> be collected by registries
 - Inform registries on their data needs early discussions
 - Process for collecting and reporting events defined / described in study protocol

Registry holders

- Consent and governance arrangements align with EU General Data Protection Regulation
- Develop policy for timely data sharing based on data protection and informed consent
- Establish a system for centralised data application requests
- Require <u>sustainable funding</u> for registries
- All
 - Transparency on access to, sharing of, and publication of data

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Core common data elements

- Participants were able to agree on core data elements to be collected
- Distinction between "must have" and "nice to have" data
- Additional data can be collected if needed to support a study
 - Needs early discussion, flexibility, agreement, registry lead-in time
 - Marketing authorisation applicants need to commit time / personnel long before approval
 - **Care**: More data ask = more registry workload & risks lower quality data



Data Quality

Recurrent concern for registry holders, MAHs/ MAAs and regulators

- Key components of quality:
 - Uniformity, representativeness, consistency, completeness, accuracy, timeliness
- Source data verification procedures needed
- Data quality control system to be established internally
- External audit to be considered
- Data quality indicators to be defined
- Similar data quality in routine collection and in registry-based studies



Parallel Regulatory HTA engagement in discussions on Post-Licensing Evidence generation

HTA Network (HTAN) reflection paper and HTAN synergy group

EMA - EUnetHTA bilateral meetings

Parallel Qualification of registries and parallel product advices

EMA research and development platform, and Focus group



Exploring HTA-Regulatory synergies: Call on a strategic level

HTA NETWORK REFLECTION PAPER ON "SYNERGIES BETWEEN REGULATORY AND HTA ISSUES ON PHARMACEUTICALS"

ADOPTED BY THE HTA NETWORK, 10 NOVEMBER 2016

- a) Pre-marketing phase
- b) Market Entry
- c) Post Marketing Real world effectiveness and safety

The Ad-hoc Synergy Group with HTA representatives (i.e. HTA Network and EUnetHTA JA3) and regulators (i.e. STAMP, HMA, EMA) is currently mapping the actions.

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Engagement through the EMA/EUnetHTA work plan 2017-2020

eunethta 13 November 2017	EUROPEAN MEDICINES AGENCY SCIENCE MEDICINES HEALTH	
EMA/661613/2017 Human Medicines Research and Development	"Late dialogues" / peri-licensing advice	
EMA-EUnetHTA three-ye 2017-2020	Gaining experience with peri-licensing advice on post-licensing data generation plans with a focus on specific products (e.g., ATMPs) or regulatory processes or tools (e.g., CMA, Adaptive Pathways, or PRIME)	Provision of parallel consultation on requirements for post-authorisation data collection plans (including registries)
	Optimise utilisation of post-licensing evidence generation for decision making Website	Collaboration in requirements for data collection and analysis of real world data including registries
17		8 June 2018



Parallel procedures in RWD settings

Qualification procedures assess the potential fitness of data derived from registry for specific types of study objectives in regulatory decision making

ECFSR registry

- HTA substantive participation as individual HTA bodies (3); also HTA observers (4)
- CHMP opinion re registry use. HTA advices drafted

EBMT registry (CAR-T) data requirements

- CHMP drafting opinion re registry use. Products under MAA simultaneously.
- HTA observers only (6+ EUnethTA); products not yet under reimbursement appraisal

Parallel advice procedures

- Post authorisation safety study protocol for product; EUnetHTA observer
- Use of RWD in post Conditional Marketing authorisation setting to expand safety and effectiveness data; HTA participation substantive as individual HTA bodies (5)



EMA research and development platform with Industry associations

EMA research and development platform with Industry associations; fully transparent/ published report and presentations

<u>Website</u>

Discussion on Post licensing evidence generation (PLEG)advices at 2nd meeting

- <u>EMA</u>
- EUnetHTA rep invited to co-present
- Outcome Focus group (EMA, Industry and EUnetHTA rep) for greater in depth understanding of barriers and issues to seeking advice on PLEG

19 Parallel Regulatory HTA procedures PLEG/RWD

8 June 2018

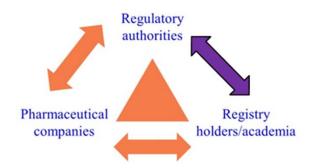


Learnings re Regulatory HTA engagement in post-licensing evidence generation

- Issues and barriers for different stakeholders in participating in PLEG advices need to be understood, be transparently & widely communicated, and addressed
- Exchange of information on processes, tools and workshops needs to continue through relevant and appropriate fora
- Foundation on which to build process for rationale PLEG evidence generation to benefit public health

How can regulators support use of disease registries?

- Methodological guidance on use of disease registries from a regulatory perspective: Likely consultation 2018
 Will address regulatory requirements and guidance for collecting / reporting AEs and ADRs
- Scientific Advice on PASS/PAES study protocol using registries, e.g. joint collaborative studies (involve HTA and payers where possible)
- Inventory of disease registries ENCePP Resources database, <u>www.encepp.eu</u>
- Facilitation of interactions between regulators, industry and registry holders during the entire life cycle of a product
- Collaboration with EU initiatives, e.g., EUnetHTA Joint Action 3, EC JRC European Platform on Rare Disease Registration



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How can regulators support use of disease registries?

EMA Qualification procedure

A voluntary scientific pathway leading to a Committee for Medicinal Products for Human Use (CHMP) opinion or a Scientific Advice on innovative methods or drug development tools

CHMP qualification opinion on the European Cystic Fibrosis Society Patient Registry

- Its current status may allow its use as a data source for regulatory purposes in studies of drugs authorised for CF (Secondary use)
 - Drug utilisation studies
 - Drug efficacy / effectiveness studies
 - Drug safety evaluation

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http://www.ema.europa.eu/ema/index.jsp?curl=pages/includes/document/document_detail.jsp?webContentId=WC500243542&mid=WC0b0 1ac058009a3dc

How can regulators support use of disease registries?

* Qualification procedure



²³ Published for consultation on the EMA website; Consultation closed 9th April 2018

Conclusions

• Paradigm shift from "product registry owned by single company" to "(joint) collaboration with disease registry for long-term patient follow-up"

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- Concerns about data quality of existing disease registries but workshops demonstrated companies and registry holders are agreeable to collaborate
- Gap between the amount/type of data collected in disease registries and data requested by regulators
 - Early regulator registry holder MAA interaction may help bridge the gap
- EU regulatory network is developing tools to support use of disease registries
- Qualification process through EMA scientific advice may provide confidence in registry data
- Activities on registries will be prioritised in the context of EMA relocation business continuity planning



Thank you for your attention

Further information

Contact us at EMAregistries@ema.europa.eu

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