European Federation of Pharmaceutical Industries and Associations

Industry Perspective on Registry-Based Studies

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EMA Workshop on registry based studies
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Examples of RWD Uses

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- To understand disease and natural history, treatment patterns, patient management optimization, impact on public health
- To support product development and utilisation, including identification and development of novel outcome measures such as digital endpoints, PROs
- To monitor risks and benefits throughout drug life cycle, effectiveness of Risk Minimization Measures, patient adherence
- To support assessments and decision making by regulators and HTA bodies (including coverage and outcomes-based payments)
- To provide insights for life science research (e.g. patient phenotypes with high unmet need; validation of novel outcome measures…)
- To increase the sustainability and effectiveness of health systems including cost-effectiveness and optimal use of products over their lifecycle

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Principles for Evidence Generation

Underpinning the development and use of medicines

Clearly and precisely identify the research question(s) or hypothesis through understanding of stakeholder needs and expectations

Identify the right data sources to provide fit-for-purpose reliable data of high quality (reuse existing data and/or new data collection)

Deploy rigorous study designs, right analytical tools and methods.
Engage with regulators and/or HTA bodies

Deliver useful, patient-centric evidence and insights to drive decisions

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Examples of RWD sources

Patient Registries

Healthcare databases including electronic health records

Observational data collected de novo (primary data)

Patient-generated health data gathered from sources that can inform on health status, e.g. mobile devices

And many more e.g. social networks, survey data, pharmacy data…

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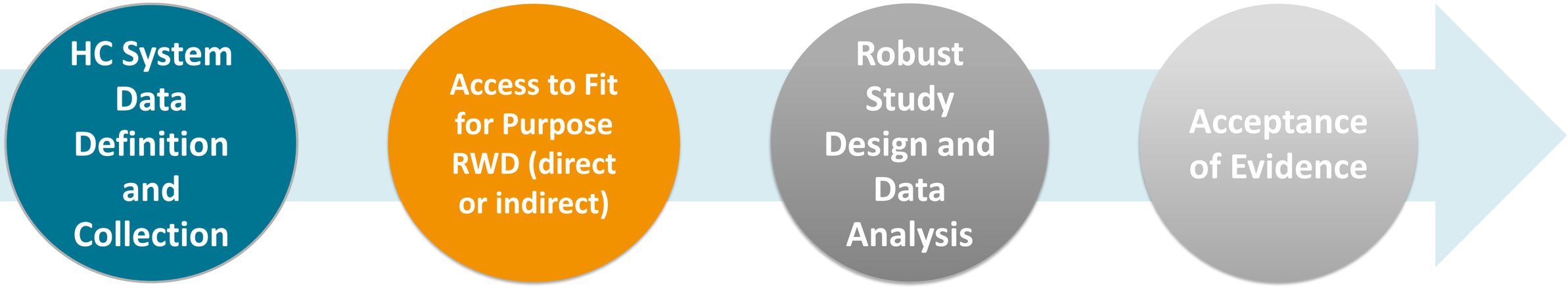
Challenges for RWD /RWE



Challenges

Major questions around relevance, depth and quality of source data

Data privacy and access issues

Familiarity with and acceptance of study designs & robust analytical methods

How to address challenges and strengthen the use of RWD/RWE

Improve the quality, relevance and interoperability of RWD source

Achieve sustainable, appropriate access and use of RWD addressing data privacy concerns (e.g. GDPR)

Develop best practices regarding study design and analytical approaches. Define plans prospectively

Drive acceptance through multi-stakeholder interactions, discussion on use cases and development of guidance

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Patient Disease Registries

Data quality

- Important to assess the quality and suitability of the data collected
- Check the breadth and depth of data collected vs. the research question
- Check the range and representativeness of patients included

Data Access

- Does registry governance allow direct or indirect access to data for MAH
  Will a third party need to perform the analyses?
- Is the registry (comprising administrators, contributors and patients) willing to partner with MAH to go further than simple data access.
  e.g. is there a willingness to add data fields / conduct quality checks and audit?

Acceptability

- Are there any concerns regarding quality and suitability?
- Does the registry have a track record of RWE acceptable to the EMA etc.
- Are the registry administrators or clinical leads able and willing to engage with feasibility reports and regulatory scientific advice processes.

This EMA guideline: initial personal reaction

Clear separation of registry best practice and registry study best practice
See also 4 disease Workshops (CF,MS, CAR-T, HAEM)

Acknowledgement of HTA as a stakeholder

Data quality

Direction to existing guidance on improving underlying quality and standardisation of registry data (see annex and *Use of patient disease registries for regulatory purposes – methodological and operational considerations; 2018*)

Data Access

Encouraging partnership with industry via clear registry governance.

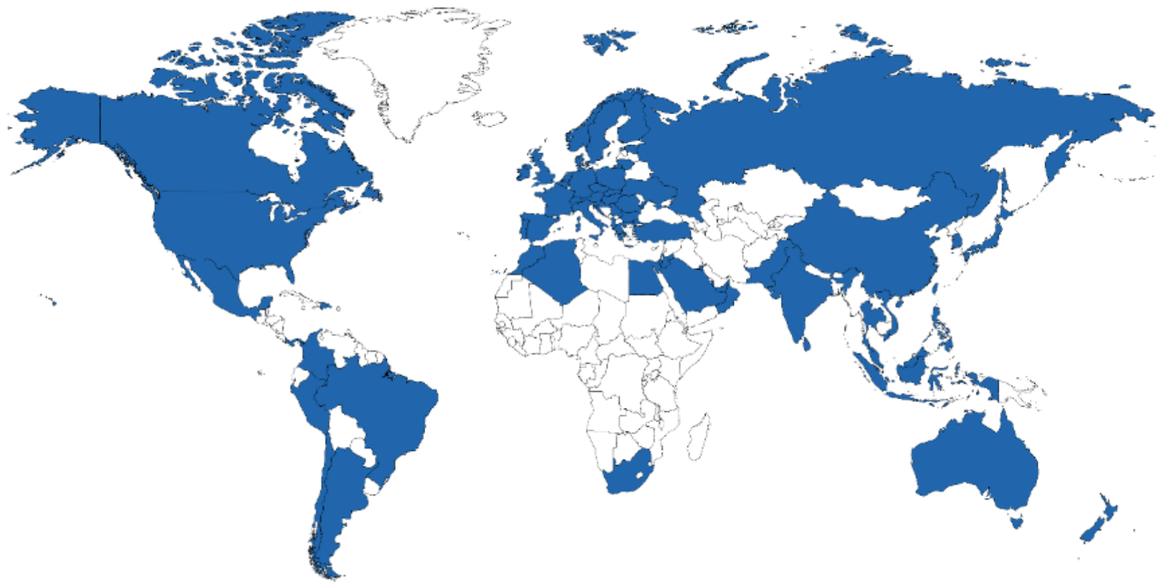
Acceptability

Clear encouragement to follow process of feasibility, advice and consultation
Obligations on MAH not weakened, so acceptability needs close cooperation with registry administrators.

Industry sponsored Patient Disease Registries

“Concerns about data quality are particularly important in the context of post-authorisation registry studies imposed to MAHs by regulators as a condition of the marketing authorisation, where the legal responsibility to conduct the study and provide valid and reliable results lies with the MAHs. This legal context has often stimulated MAHs to create their own product registry providing them full control of the data collection.” Use of patient disease registries for regulatory purposes – methodological and operational considerations section 5.6.1 / 2018

Sanofi Genzyme Rare Disease Registries
Participating countries worldwide



- Presence in **67 countries** worldwide
- More than **17,000 patients** enrolled
- Over **900 participating sites**
- Support from **1,200 Healthcare Professionals**

GAUCHER

64	countries
6,520	patients
270	sites
41	manuscripts published

MPS I

39	countries
1,220	patients
130	sites
12	manuscripts published

FABRY

48	countries
7,230	patients
240	sites
24	manuscripts published

POMPE

42	countries
2,090	patients
230	sites
6	manuscripts published

As of Nov 2019

SANOFI GENZYME

Opportunities to Work with Industry

- Industry is willing to fund ongoing disease registries, not just product registries, and not just to meet post launch commitments…
- Industry can provide levels of resources and expertise to maintain a “regulatory standard” registry.
- Critical to maintain close links with the clinical and patient communities
- Opportunity to transfer knowledge (of managing registries to the expected regulatory standard, and of scientific advice processes) to third party registries as part of trusted partnerships

How to move forward?

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RWD / RWE has **great potential to improve patient lives their experience of care and to increase the efficiency of the healthcare system**.

- **Collaboration** across stakeholders is needed
- Everything needs to be **connected and aligned**:
  - **EFPIA's vision for health information infrastructure:** to have well-resourced healthcare organisations designed to enhance delivery of quality care and to provide high quality RWD to accessible research platforms
  - **EFPIA's vision for evidence generation:** to increase collaboration to develop high quality data and research networks that allow the integration and interoperability of data from a wide range of sources
  - **EFPIA's vision for key decision-making processes:** to generate evidence and insights based on RWD using a variety of designs and methodologies which are fit for purpose and accepted by relevant stakeholders

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Thank you!






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