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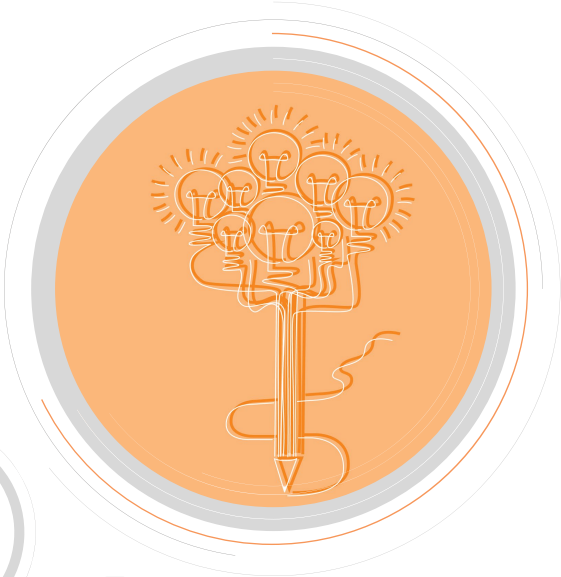

Promote use of high-quality real-world data (RWD) in decision making

Underlying actions

EMA's Regulatory Science Strategy to 2025 – Human Stakeholder Workshop

Chaired by Sabine Straus, PRAC, Violeta Stoyanova-Beninska, COMP on 18 November 2019
Presented by Peter Arlett, Head of Pharmacovigilance and Epidemiology, EMA

Disclaimer

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Promote use of high-quality real world data (RWD) in decision-making

- Create a sustainable, quality assured, flexible framework delivering rapid access to and analysis of representative, longitudinal RWD throughout a product's lifecycle
- Develop a capacity that will enable the Agency to rapidly and securely access and analyse large amounts of healthcare data
- Accelerate the implementation of a learning regulatory system based on electronic health records and other routinely collected RWD

Proposed rewording of core recommendation

From

“Promote use of high-quality real-world data (RWD) in decision making”

To

“Start an initiative where the regulatory system learns based on submissions of big data (including RWD): to include submissions from industry through the product lifecycle, tracked for outcome and learnings shared with stakeholders and the development of guidance”

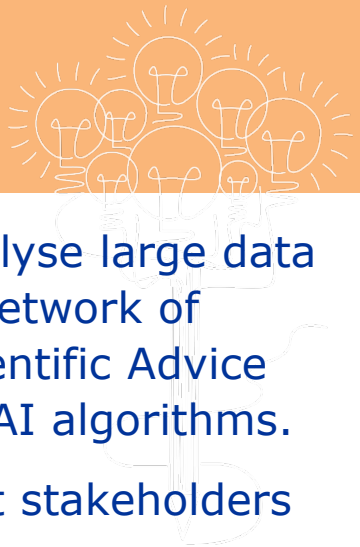
Create a sustainable, quality assured, flexible framework delivering rapid access to and analysis of representative, longitudinal RWD throughout a product's lifecycle

- Build on ongoing efforts (in EU and internationally) to provide clarity on scope and quality of sources of RWE, recognising governance and resources required for these sources and identifying where gaps exist. The EMA and HMA could also partner with the European Commission to develop a unified approach on the collection, curation and interoperability of health data and establishment of a European health data resource base for the benefit of European citizens.

Create a sustainable, quality assured, flexible framework delivering rapid access to and analysis of representative, longitudinal RWD throughout a product's lifecycle

- Demonstration projects are essential to increase knowledge, capacity and confidence levels amongst RWD stakeholders including pharmaceutical companies and regulators.
- Enhanced acceptability of RWD to support regulatory decisions must also involve and evolve with patients, HTA bodies, healthcare professionals and other stakeholders.

Develop a capacity that will enable the Agency to rapidly and securely access and analyse large amounts of healthcare data

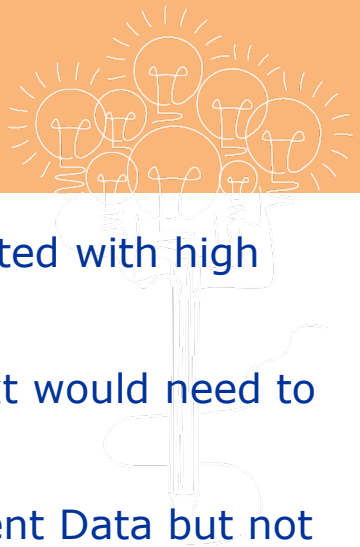


- Includes building computing capacity to receive, manage and analyse large data sets including patient level data (PLD); establishing a federated network of analytics centres linked to regulatory agencies; strengthened Scientific Advice that can advise on Big Data, and; the Network ability to validate AI algorithms.
- Such a capacity should be developed in consultation with relevant stakeholders including industry.
- The EMA should perform analysis in house on raw data while ensuring the independence and integrity of the process. Such analyses should be available to 3rd parties.

Accelerate the implementation of a learning regulatory system based on electronic health records and other routinely collected RWD

- Launch a strategic initiative to integrate RWE in drug development, including the use of demonstrator projects to engender familiarity.
- A dedicated EMA RWE pilot program in which regulators and sponsors can publicly share lessons learned (with protections for confidential commercial information) for the benefit of all stakeholders, which will improve the quality of RWE submissions in the future.

Develop the capability to use Individual Patient Data

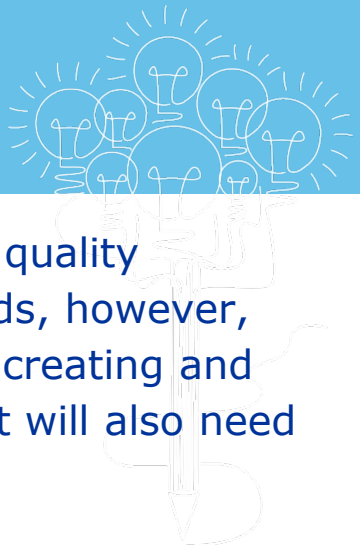


- Any plans to (re-)analyse clinic trials individual patient data is associated with high investment within the regulatory network.
- Possible/potential roles of NCA's assessments capacities in this context would need to be explored in a timely manner
- EMA should focus on understanding and reviewing the Individual Patient Data but not re-analysing data in-house.

Establish an EU Data Quality and representativeness framework

- To include guidelines, a data standardisation strategy, a strengthened process for Scientific Advice data qualification, and promotion across the Member States of uptake of electronic health records, data linkage and secure data availability.
- Build on ongoing efforts (in EU and internationally) to provide clarity on scope and quality of sources of RWE, recognising governance and resources required for these sources and identifying where gaps exist.

Establish an EU Data Quality and representativeness framework



- To ensure "high quality" RWD, internationally aligned fit-for-purpose quality requirements for regulatory purposes are essential. Beyond standards, however, this discipline also needs quality management in practices related to creating and using RWE sources. Establishing best practice in quality management will also need pilots to advance practice.

Ensure the EU Regulatory network has the expertise to regulate product dossiers including real world data

- Includes: development of a Big Data training curriculum and strategy based on a skills analysis across the Network; deliver collaboration with external experts including in academia, and; targeted recruitment including data science, biostatistics, epidemiology, advanced analytics and AI + strengthen EMA working groups with new expertise on real world data.
- Continuing education resources to enhance reviewers' consistent understanding of novel RWD source types, RWD quality considerations, and evolving analytical methodologies for generating RWE (especially methods applied to observational data).

Collaborate with stakeholders in Europe and internationally to leverage ongoing initiatives and share best practice on RWE

- Includes development of guidelines at multilateral fora; contribution to data standards through standards bodies; bilateral collaboration and sharing of best practice with international partners + establishing an EU big data 'stakeholder implementation forum').
- EMA/EC are encouraged to organize fora to align on design, collection and use of RWD with regulators and HTAs.

Collaborate with stakeholders in Europe and internationally to leverage ongoing initiatives and share best practice on RWE

- Regulators, industry and other stakeholders need to engage widely to help establish momentum for appropriate use of RWE. Workshops are one mechanism that has worked in other domains for regulatory change and could be used for this purpose.

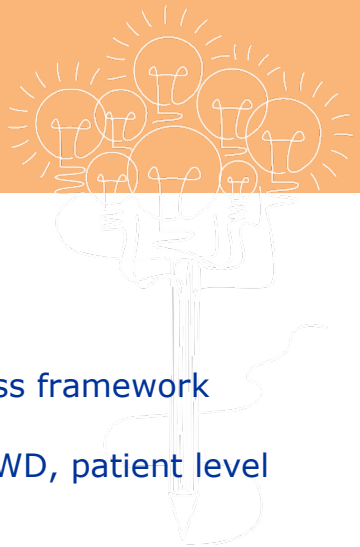
Collaborate with stakeholders in Europe and internationally to leverage ongoing initiatives and share best practice on RWE

- EMA should remain active in this field of research internationally.
- Seek to align and contribute to extend the standards and methodologies for collecting, analysing and validating RWE use internationally.

Deliver secure ethical patient-focussed governance for accessing, managing, analysing, and assessing real world data

- Collaborate with regulatory authorities, especially Data Protection Agencies, and where appropriate European Data Protection Board (EDPB) to facilitate a harmonised approach regarding the use of RWD/big data, especially the re-use of data for secondary purposes, and diminish barriers that might hamper big data use.
- Seek to align and contribute to extend the standards and methodologies for collecting, analysing and validating RWE use internationally.

Promote use of high-quality real world data (RWD) in decision-making



Pre-consultation

- Create framework for rapid access to and analysis of RWD throughout lifecycle (*network, data sharing platform + governance and funding*)
- Develop a capacity for Agency to access and analyse large amounts of healthcare data (*computing capacity and skills*)
- Implement a learning regulatory system based on RWD (*processes to feed data into assessment*)

Consultation *additions*

- RWD learnings initiative
- Data quality/ representativeness framework
- Build computing capacity for RWD, patient level data, AI: analytics centres
- Regulatory capacity for studies
- Skills: training curriculum and strategy
- International collaboration and stakeholder dialogue
- Governance – clarity on data protection

Any questions?

Further information

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