



FONDAZIONE
PER LA RICERCA FARMACOLOGICA
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**XIV FORESIGHT TRAINING
COURSE**
*The health emergency: regulatory
crash and future perspectives*

Real use of real-world data

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Study on the use of real-world data (RWD) for research, clinical care, regulatory decision-making, health technology assessment, and policymaking

Final report and recommendations

Purpose of the study



Explore the value of existing RWD initiatives and outputs

- To carry out a mapping exercise of completed and ongoing initiatives across five main use cases of RWD
- To provide an overview of the main objectives and outputs of these RWD initiatives



Identify the challenges to RWD use and best ways to address them

- To identify the key barriers to RWD use and main stakeholders' needs
- To provide a comprehensive overview of EU-funded research projects and their outputs
- To assess the extent to which these existing initiatives and their outputs address the challenges to RWD use



Realise the potential of RWD

- To provide an overview of existing and potential complementarities between RWD research projects
- To identify unmet RWD research needs along the health care value chain
- To derive policy recommendations on the use of RWD addressing mutual needs of various stakeholder groups

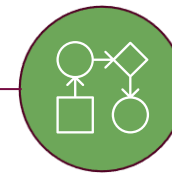
Three phases of methodology

Scanned over 35 000 projects, 460 000 monitoring documents and 120 000 publications in EC/medical databases and project websites



Data collection phase

Data analysis phase



Mapped 192 RWD initiatives and 579 research outputs through extensive desk research and interview programmes

Conducted 6 workshops with over 200 stakeholders and interviews with high-level EU officials



Data validation phase

Key activity areas

Five use cases of RWD

Research

Research activities focused on investigating human health by involving RWD from basic research to pre-clinical and clinical research, including clinical trials/investigations of health technologies

Clinical practice

Health care activities focused on the use of established health technologies under standard and common procedures which are implemented in line with RWD-based clinical guidelines

HTA

Decision-making activities focused on assessing the use of health technologies in terms of relative effectiveness, costs-effectiveness and other criteria like the burden of disease based on RWD

Regulatory decision-making

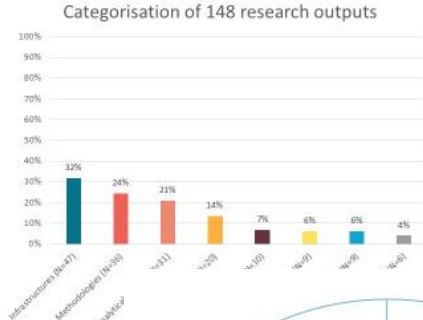
Regulatory decision-making activities focused on evaluation and supervision of medicinal products based on RWD across the pre-market, market authorisation and post-market phases

Policy-making

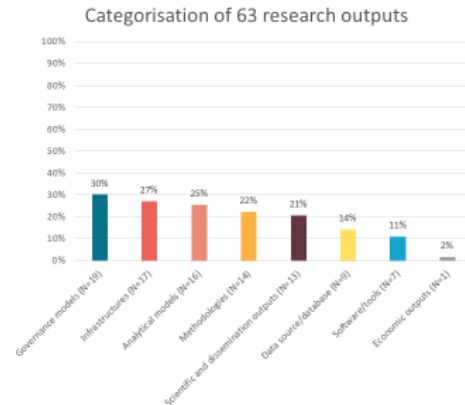
Policy decision-making activities focused on design and implementation of decisions, plans, and actions using RWD to achieve specific population health goals

5 use-case: key projects & outputs

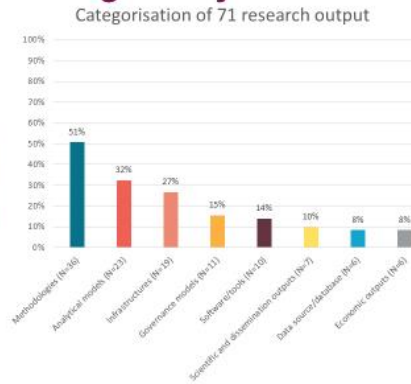
Clinical practice use case



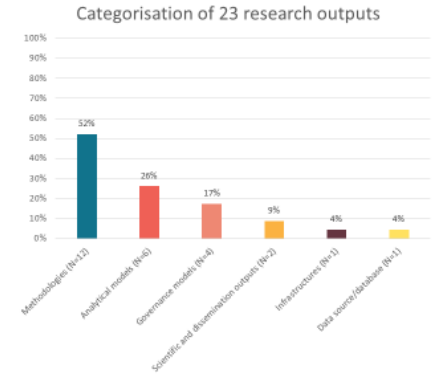
Policy-making use case



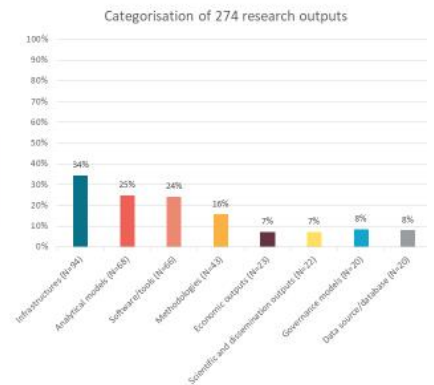
Regulatory use case



HTA use case



Research use case



Main barriers to the use of RWD and stakeholders' needs

- **Barriers hamper stakeholder capacity to exploit the potential of RWD in health**
- **Completed and ongoing initiatives address these barriers to some extent only**

Operational level

- Regulatory fragmentation
- Uncertainty on GDPR



Technical level

- Lack of semantic and technical interoperability
- Lack of common data standards

Methodological level

- Limited guidance on appropriate methods for data collection and analysis

Complementarities and synergies across RWD projects

- Current focus of RWD initiatives is primarily on proof of concepts and pilot applications to particular settings
 - Translational, pre-clinical and clinical research 
 - HTA, regulatory decision-making and policymaking 
- Few examples of synergies and exploitation of earlier outputs between funded initiatives
- Next steps should revolve around demonstration projects with wider relevance and broader use of RWD

Recommendation I

Develop common methodological principles
for RWD collection and analysis

Potential actions:

- To support research on the development and testing of new statistical analytical methods for various types of RWD and fields of application
- To support actions aimed at finding an agreement on common methodological principles, guidelines, reporting instructions and particularities of using them for specific questions and RWD types
- To support training courses for users of RWD on methodological tools, principles and practices for RWD analysis, covering different types and use cases of RWD

Recommendation 2

Support the accessibility and interoperability of RWD

Potential actions:

- To support testing of existing data models and exchange standards or pilot new ones
- To support further work, including establishing Electronic Health Record Exchange Formats for other types of RWD sources under the work programme
- To support pilots on machine learning techniques to solve the issue of interoperability
- To encourage open data sources as outputs of EU-funded RWD projects
- To further encourage the adoption of FAIR
- To study how RWD can be more widely used to inform decision-making for regulatory decision-making, HTA and policymaking purposes across the Member States

Recommendation 3

Ensure a coherent interpretation and application of the GDPR for the use of RWD

Potential actions:

- To explore best practices for compliance with the GDPR regarding health RWD use and put it widely available
- To support training courses for GDPR application for concerned stakeholders

Recommendation 4

Improve information on and understanding of RWD's scientific integrity, quality, and accuracy

Potential actions:

- To explore and test data quality standards in the context of its various usages
- To support pilot cases to establish transparent reporting guidelines for RWD, which would address issues related to data sources, data collection methods, data structure, and missing data
- To explore and test tools to estimate data accuracy
- To support training courses on data entry for data sources that are frequently used for RWD analysis
- To explore incentive models to support enlarged and accurate data entry by individual benefits

Recommendation 5

Strategy for RWD use in health care

Potential actions:

- To consider actions on RWD use more widely to stimulate the involvement of less represented stakeholders in RWD initiatives and to ensure a more balanced representation of all Member States in such initiatives
- To support stakeholder forums to exchange experiences on RWD use
- To support the establishment of sustainable RWD infrastructures
- To encourage broader stakeholder representation in particular patient organisations and broad Member State coverage in future RWD calls in Horizon Europe and EU4Health
- To support and link the existing efforts to address cross-cutting challenges in health care relating to data quality and GDPR application with other ongoing EU-wide developments, such as the development of the European Health Data Space and European Open Science Cloud

Recommendation 6

Demonstrate RWD's practical relevance through pilot actions

Potential actions:

- To support dissemination fora for RWD projects to explain and exchange findings
- To support training for decision-makers, practitioners, and patients on the use of RWD
- To research pilot cases of high value/good practices for the use of RWD
- To explore trusted models for data governance and use
- To encourage research to further evaluate the usability/practical relevance of previously funded projects
- To support open-source methods
- To improve trust in the use of RWD
- To improve understanding of how RWD are protected, shared, and used
- To consult the public and professionals about how data are used
- To support research in experimental AI pilots for validation of AI tools

Recommendation 7

Promote synergies between existing RWD initiatives and exploit work of completed projects

Potential actions:

- To encourage the exploitation of prior findings and outputs
- To highlight best practice/relevant project examples in any new calls and ask for planning and funding of liaison activities
- To encourage dissemination for ongoing projects to exchange best practices
- To increase the adoption of FAIR data principles, including their acceptance and implementation

Findings presented here derive from the **Study on the use of real-world data (RWD) for research, clinical care, regulatory decision-making, health technology assessment, and policy-making** [European Commission, Directorate-General for Research and Innovation; written by PPMI, Maastricht University and Fondazione per la Ricerca Farmacologica Gianni Benzi Onlus; available at <https://op.europa.eu/en/publication-detail/-/publication/6f758166-2198-11ec-bd8e-01aa75ed71a1/language-en>]



Thank you