

# DEVELOPMENT OF A REAL WORLD EVIDENCE STUDY DESIGN TOOL: RWE FRAMEWORK

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## BACKGROUND AND OBJECTIVES:

- A holistic approach to healthcare — in which data obtained from a variety of sources can complement traditional clinical trials to aid treatment decisions — is increasingly recognised to be important and an area where real-world evidence (RWE) can offer valuable insights.
- Many considerations face teams planning RWVE studies, including an array of available real-world data sources and different RWE study designs.
  - These choices can present a challenge to researchers when selecting the optimal study design to address their research objectives.
  - Team members may also have different levels of familiarity with RWE best practices, limited time and/or limited resources available to plan their studies.
- The main objective of this analysis was to develop a holistic and concise tool to encourage a methodical approach to RWE study planning and implementation.

## METHODS:

### Literature review

- A literature review was performed to identify publications with decision tools and/or visual aids that describe best practices for planning and/or conducting RWE studies.
- Articles with English abstracts were identified through Embase and MEDLINE database searches.
  - Publications dated from 2008 to 2017 were included.
  - The search terms 'real world evidence' or 'real world data' or 'pragmatic trial' were combined with 'consensus statement' or 'flow diagram' or 'decision tool' or 'best practice' or 'generation' or 'algorithm'.
  - Comments, editorials, letters, case reports, legal cases, lectures, news, technical reports, and publications focussed on children were excluded.
- Supplemental online searches were conducted via Google.com and websites associated with relevant professional organizations, government institutions and public-private partnerships.<sup>[1-6]</sup>

### Development of RWE Framework

- The conceptual framework was developed to aid the planning and design of RWE studies using a staged approach.
  - Key components of a best practice RWE study planning process were identified and organised in a logical and chronological sequence to create a flow diagram.
  - An infographic design and instructions based on a Q&A framework were developed to guide users through the flow diagram.
  - The infographic RWE Framework was further refined following feedback from Internal Advisory Panels.

### Pilot testing

- The initial design of RWE Framework was pilot tested by 40 RWE professionals from 9 European countries via two group workshops before finalisation.
  - Professionals represented areas of RWE, Medical Science, Drug Safety, Epidemiology, Regulatory, Research and Development and Market Access.
  - RWE Framework was tested via a series of group exercises based on case studies across settings including pain, asthma, diabetes and osteoarthritis.

## RESULTS:

### Literature review

- 371 abstracts were identified; title and abstract screening was completed by a single assessor: 3 articles were selected for detailed review.
  - Most articles focused on a subset of RWE study types, such as pragmatic trials or database studies, or on a subset of RWE objectives, such as evaluating clinical effectiveness.
  - No published RWE study design decision tools or visual aids were identified from the Embase/MEDLINE searches.
- Only two online sources included decision tools or visual aids: one a detailed interactive online resource RWE Navigator<sup>[7]</sup> and the other a brief illustrative diagram from the ABPI Guidance<sup>[8]</sup> on the definition, use and practical issues surrounding the generation of RWE.
- A gap was identified for a concise tool that supports decision making on RWE study design.

### Conceptual Framework and Pilot Test

- The pilot test exercises with RWE professionals generally provided positive feedback on the value of the RWE Framework and its ease of use. Recommended changes included:
  - Refined flow and sequence of the framework components, such as the starting point and next steps in the flow
  - Labelling the stages in the framework
  - Additional steps for inclusion in the framework
  - Listings of items under key steps, e.g. types of data of interest
  - Supporting information content, e.g. terminology descriptions
  - Infographic design improvement, e.g. use of icons

### RWE Framework

- The newly developed tool (Figure 1) captures a multi-step decision process to determine an optimal approach to study planning and design, including consideration of:
  - Research objective(s)
  - Product approval status
  - Study conduct (routine practice vs intervention)
  - Outcomes of interest
  - Data availability in routine practice
  - The need for primary data collection and randomisation
- The tool guides on appropriate study type and methodology (covering options under the categories of retrospective, prospective observational and pragmatic trial) and applicable regulatory standards.
- Instructions and checklists lead users through the planning exercise step-by-step, as well as to provide background information on key definitions.

## CONCLUSIONS:

- The RWE Framework is a novel and visual tool which was designed to address an unmet need for a concise, easy-to-follow aid that can inform RWE study planning for a broad range of RWVE study types and research objectives.
- RWE Framework can enhance RWE decision-making involving multiple stakeholders and replace ad-hoc RWE study planning processes with a robust approach.
- The future use of RWE Framework as a visual aid for planning RWE studies should be complemented with additional resources, including in-depth knowledge regarding the product and/or therapy area.
- Further testing of RWE Framework across a range of disease areas and decision-making scenarios is warranted, along with potential development of some therapy-area specific tools.

### References:

- European Medicines Agency ([www.ema.europa.eu](http://www.ema.europa.eu))
- The US Food and Drug Administration ([www.fda.gov](http://www.fda.gov))
- The Association of the British Pharmaceutical Industry ([www.abpi.org.uk](http://www.abpi.org.uk))
- The International Society for Pharmacoeconomics and Outcomes Research ([www.ispor.org](http://www.ispor.org))
- Get Real ([www.imi-getreal.eu](http://www.imi-getreal.eu))
- Network for Excellence in Health Innovation ([www.nehi.net](http://www.nehi.net))
- RWE Navigator ([www.rwe-navigator.eu](http://www.rwe-navigator.eu))
- Demonstrating value with real world data: a practical guide. <http://www.abpi.org.uk/about-us/resources/publications-library/real-world-data>

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Figure 1: RWE Framework

