IDERHA Task 6.4 Regulatory and HTA stakeholder workshop 30.10.2023 - Executive summary

On 30th October 2023, IDERHA's Work Package 6 held its first workshop with experts from regulatory authorities and HTA organizations. The long-term goal of this work is to develop recommendations on criteria for determining the acceptability of real-world evidence (RWE) for key public health decision-making (i.e., regulatory and HTA). The first step towards this goal is a landscape review of the best practices and remaining gaps in current policies regarding the use of RWE. This workshop was convened to solicit expert input on landscape review and identify priority areas that require development to inform the future policy recommendations.

Key Takeaways

- Participants commented that the project goals and the landscape review are timely given the quickly evolving policy environment and expanding interest in the use of real-world evidence to support regulatory and HTA decision-making.
- Identifying areas of alignment across different policies and countries will help encourage efficiencies.
- Separate analysis may be required between regulatory and HTA documents, but it is also important to highlight where there is alignment and divergence between them where possible.
- Guidance for both pharmaceutical and medical devices should be considered with the consideration that there are likely to be differences.
- A range of topics were identified as potential gaps in current guidance that need of further exploration, including:
    - Uncertainties with how to analyse and apply different types of emerging RWE (e.g. data from AI, patient wearables)
    - Lack of trust and understanding of RWD and RWE undermining further adoption (e.g. cross-jurisdictional use and acceptability of RWE)
    - Developing good study conduct practices around the collection and use of RWE (e.g., concerns of governance and transparency)
- If possible, continue to update the review over time to incorporate guidance that comes out across the span of the IDERHA project.

IDERHA Task 6.4 Regulatory and HTA stakeholder workshop 30.10.2023 - Full report

Introduction

On 30th October 2023, Work Package 6 (WP6) of the IDERHA project held its second multi-stakeholder workshop with five experts from regulatory and health technology assessment (HTA) organisations (Appendix 1). The long-term goal of this work is to develop recommendations on criteria for determining the acceptability of real-world evidence (RWE) for key public health decision-making (i.e., regulatory and HTA). The first step towards this goal is a landscape review of the best practices and remaining gaps in current policies regarding the use of RWE. This workshop was convened to solicit expert input on landscape review and identify priority areas that require development to inform the future policy recommendations.

The workshop aimed to achieve the following objectives:

- Familiarise key stakeholders with IDERHA and the policy activities being undertaken within the project
- Engage with regulators and HTA to:
    - Provide input on the landscape review processes
    - Share insights on their policy priorities

During the workshop, there was an overview of the IDERHA project and the project aims to change how health data can be used, accessed, and shared. This was followed by a presentation of the landscape review's research protocol, current status and preliminary findings. The landscape review is focused on analysing the current policies, guidelines, and frameworks for use of RWE within regulatory and HTA settings. The overview included details on the research objectives, document inclusion criteria, the list of the current organisations and initiatives whose policy or guidance documents were included in the review, the analysis extraction categories, and a summary of the current findings.

Participants were asked to provide feedback on both the review process and emerging areas that are not covered in sufficient detail, or at all in the documents. The interactive approach of the workshop aimed to inform the WP6 activities by incorporating the perspectives of the workshop participants.

Value of Building Consensus

The participates agreed that given the steady increase in recent years of new policy of the use of RWE for decision-making, the timing of the project and need to build consensus would be beneficial. There was also general agreement that it will be important to identify areas of alignment among policy documents. In particular, to increase agreement on

foundational work such as definitions for terms such as RWE, data quality, and data governance.

Extensiveness of review sources

The participants agreed that the list of organisations with policies or guidance documents included in the review (Appendix 2) were comprehensive. There were recommendations to consider documents that may be out of scope due to their broader focus but could be informative such as:

- The World Health Organization (WHO) – including a paper on the use of RWE in the field and a paper on the use of artificial intelligence (AI).
- IMI-Get Real initiative/GetReal Institute
- HTAi Global Policy Forum background paper and journal publication, 2019

It was acknowledged that some of the documents, particularly from WHO, may be out of scope of the inclusion criteria due to their broader focus.

There was a brief discussion on the inclusion of tools and checklists that may include transparency requirements. It was noted that these may not be in scope of the current inclusion criteria, but the team agreed to follow-up on the recommendation.

Feedback on Review Analysis Approach

i. Regulatory vs HTA

There was a consensus among the group that given the distinct applications of RWE in regulatory and HTA contexts, the guidance required is likely to vary. Therefore, it is appropriate to analyse these documents as separate groups.

Participants stated a clear interest in understanding where there may be alignment or convergence in policies. The ability to identify 'best practice' would mean that efficiencies can be recognised and identified for broader adoption (e.g. shared outcomes of interest, resource utilisation, or safety data).

ii. Medicines vs Devices/Medtech

Similar to the feedback above, the group indicated the likelihood of different requirements for medicines and medical technologies but were eager to identify areas of alignment and divergence. Participants were eager to understand potential interlinkage between medicines and medical devices, particularly in areas like advanced therapeutics such as combination products, cell and regenerative therapies. Examining the scope of how RWE has been used in drugs, biologics, or medical devices will be important. It was noted that RWE has been used for some time in medical devices.

iii. Continuing Evolution of Policies

It was suggested that ongoing updates to the review over time would be beneficial to ensure newly published policy and guidance documents are incorporated and to assess the evolution of these documents.

Current Policy Gaps

Participants identified several gaps or limitations in current RWE policies which can be broadly split into the categories below.

How to analyse, validate and apply different types of RWE data examples:

- AI generated data, apps and personal/wearable device derived data
- Synthetic data
- Patient experience data

Ability to use RWE across-jurisdictions examples:

- Cross-jurisdictional collection of real-world data: Especially relevant in cases of rare diseases where there are small populations, and an international database of real-world data could be beneficial.
- Acceptability of data from other countries: determining whether data from other countries would be considered generalizable.

Advancing research practices and tools examples:

- Governance and good research practice:
- Tokenisation: linking data together across datasets and the potential impacts on privacy

Procedural Guidance examples:

- Incorporating qualitative data in submissions

While participants were not asked to prioritise these gaps or limitations, AI was mentioned as a particular concern due to its ever-evolving nature.

Conclusions

The workshop highlighted the importance and timeliness of the landscape review for both regulatory and HTA stakeholders. There is a clear need to understand where there is alignment in current policies, especially when considering regulatory versus HTA and medicines versus medical devices, to encourage efficient and systematic structures for use and analysis of RWE. There is also a desire to identify disparities in current policies and where there is a lack of consensus.

Areas with potential limitations or gaps in policy or guidance include how to analyse, validate, and apply different types of RWE, such as AI-generated data, cross-jurisdictional use of RWE, and procedures surrounding the collection and use of RWE.

Recommendations

- Identifying areas of alignment across different policies and countries will help encourage efficiencies.
- Separate analysis may be required between regulatory and HTA documents, but it is also important to highlight where there is alignment and divergence between them where possible.
- Guidance for both pharmaceutical and medical devices should be considered and where possible differences should be highlighted.
- A range of topics were identified as potential gaps in current guidance that are in need of further exploration including:
    - how to analyse and apply different types of emerging RWE e.g. data from AI, patient wearables
    - lack of trust and understanding of RWD and RWE undermining further adoption e.g. cross-jurisdictional use and acceptability of RWE
    - Good study conduct and practices around the collection and use of RWE, including concerns of governance and transparency
- If possible, continue to update the review over time to incorporate guidance that comes out across the span of the IDERHA project.

Appendix 1 - Workshop Attendees

Regulatory and HTA stakeholder Participants

Name	Organisation	Role
Patrice Verpillat	European Medicines Agency (EMA)	Head of RWE workstream
Dan Ollendorf	Institute for Clinical and Economic Review (ICER)	Chief Scientific Officer and Director of HTA Methods and Engagement
Vandana Ayyar-Gupta	National Institute for Health and Care Excellence (NICE)	Scientific Adviser, Data and Analytics Team
Simon Singer	Australian Therapeutic Goods Administration, (TGA)	Principal Medical Adviser, Medical Devices Authorisation Branch
Amanda Cuss	Australian Therapeutic Goods Administration, (TGA)	Principal Medical Adviser, Medical Devices Surveillance Branch

IDERHA team member participants

Name	Organisation
Fabio Bisordi	Roche
Ravinder Claire*	National Institute for Health and Care Excellence
Heather Colvin*	Johnson & Johnson
Katharine Cresswell*	National Institute for Health and Care Excellence
Morten Deleuran Terkildsen	DEFACTUM
Dipak Kalra	iHD
Rebecca Lumsden	Sanofi

Rita Peeters*	Johnson & Johnson
Rose Purcell	Takeda
Susan Sandler	Johnson & Johnson

*Speaker

Appendix 2 – List of organisations with documents included in landscape review