

All information regarding future IHI Call topics is indicative and subject to change. Final information about future IHI Calls will be communicated after approval by the IHI Governing Board.

Topic 2: Development of practical guidance and recommendations for using real world data/real world evidence in healthcare decision-making

Expected outcomes

Industry, sponsors, and other stakeholders have access to practical guidance and recommendations on the use of real-world data/real world evidence (RWD/RWE)¹ that could be followed to support the development, regulatory, Health Technologies Assessment (HTA), and payer decision-making of innovative medicines and health technologies with a focus on drug, drug-device combinations and medical devices.

Regulators, HTA bodies and payers have relevant, robust evidence on existing use cases on the suitability of RWD/RWE to support their decision making.

Scope

The use of real-world evidence to support decision making on the safety of drugs and medical devices is already well established. More recently, RWE has also been used to complement evidence and support marketing authorisation, conformity assessments and HTA submissions, however, these submissions are usually custom-made to a specific use-case. Preparing and assessing each submission requires significant expertise and effort from both the sponsor and the healthcare decision maker.

To streamline this process and facilitate the efficient use of RWD & RWE to better inform healthcare decision making, a broadly accepted, practical guidance is needed.

To address this challenge, the action funded under this topic should:

- Actively align and complement other relevant initiatives including the European Medicines Agency's vision to establish the value of RWE across the spectrum of regulatory use cases by 2025.²
- Identify the main challenges faced by industry, sponsors, and other stakeholders in the routine use of RWD/RWE for regulatory and HTA decision-making.
- In collaboration with the relevant stakeholders, identify, review, and evaluate existing methodologies, guidelines, and practices for the use of RWD/RWE in healthcare decision making. A particular focus should be regarding an in depth review of a broad range of use cases where RWD/RWE has been previously assessed for decision-making for drug, device, and drug-device combinations. This review should include methods, designs and characterisation of RWD/E and map the features that enable efficient assessments.

¹ Real World Data (RWD) are defined as "routinely collected data relating to a patient's health status or the delivery of health care from a variety of sources other than traditional clinical trials." Real-world evidence (RWE) is defined as the information derived from analysis of RWD. <https://doi.org/10.1002/cpt.1426>

² <https://doi.org/10.1002/cpt.2479>

- Develop a draft of the practical guidance and recommendations on the use of RWD/RWE to support decision making processes.
- Test the draft guidance in several pilots :
 - The precise scope of these pilots should be selected by the full consortium during the full proposal preparation and should address multiple contexts and areas that are not already being addressed, focusing but not limited to, on chronic, serious diseases, oncology, auto-immune. They should also cover development and the regulatory, HTA, payer assessment of drugs, devices and/or drug-device combinations.
 - The pilots should include one for post authorisation safety studies.
- Based on the learnings from the pilots , finalise the practical guidance and recommendations on the use of RWD/RWE to support drug/device development, regulatory, HTA and payer decision making processes.
- Broadly disseminate the guidance and recommendations to the stakeholder community.

Applicants are expected to develop a strategy and interaction plan for generating appropriate evidence as well as engaging with regulators, HTA agencies and payers in a timely manner (e.g., through national competent authorities, the EMA Innovation Task Force, qualification advice).

In addition, while the project will focus on supporting the development of a practical focused guidance, the funded project is also expected to explore synergies with complementary initiatives to advance RWD/RWD in Europe such as the GetReal Institute, REDDIE, More-EUROPA, Oncovalue, Real4Reg, RWE4Decisions, and REALM³. It should also be aligned with the ambitions and guidelines set out for the European Health Data Space (EHDS)⁴.

Expected impacts

The action under this topic is expected to achieve the following impacts:

- Improved access to innovations that meet the increasing diverse need of patients and those of the healthcare systems.
- Better informed decision-making at different levels of the healthcare system (authorities, organisations) using RWD/RWE that will in turn contribute to a better allocation of resources towards cost-effective innovations as well as representation of different patient populations and needs.
- Faster entry to the market of cost-effective drug/device/diagnostic & combination products developed by industry, which could translate to a positive effect on their R&I investments.

Why the expected outcomes can only be achieved by an IHI JU action

Translating current RWD/RWE standards into a practical guidance that can be accepted and implemented by decision-makers is a significant challenge. The active involvement of many stakeholders working collaboratively in partnership is needed to ensure such a guidance has broad applicability and adds value to the broader initiatives already underway. The diverse nature of these stakeholders, which includes patients, real world data custodians, academics, and SMEs with expertise in RWD, industry drug and device developers, regulators, HTA agencies, and payers, means that a public-private partnership is the ideal framework for such a collaboration.

³ www.getreal-institute.org, www.reddie-diabetes.eu, cordis.europa.eu/project/id/101095479, oncovalue.org, www.real4reg.eu, realm-ai.eu

⁴ health.ec.europa.eu/ehealth-digital-health-and-care/european-health-data-space_en

Identified industry consortium

In the spirit of partnership, and to reflect how IHI JU two-stage call topics are built upon identified scientific priorities agreed together with a number of proposing industry beneficiaries (i.e. beneficiaries who are constituent or affiliated entities of a private member of IHI JU), it is envisaged that IHI JU proposals and actions may allocate a leading role within the consortium to an industry beneficiary. Within an applicant consortium discussing the full proposal to be submitted for stage 2, it is expected that one of the industry beneficiaries may become the project leader. Therefore, to facilitate the formation of the final consortium, all beneficiaries, affiliated entities, and associated partners are encouraged to discuss the weighting of responsibilities and priorities regarding such leadership roles. Until the role is formalised by execution of the Grant Agreement, one of the proposing industry beneficiaries shall as project leader facilitate an efficient drafting and negotiation of project content and required agreements.

Indicative budget

- The maximum financial contribution from the IHI JU is up to EUR 14 000 000. ***NB: this amount is indicative and subject to change, pending approval by the IHI Governing Board.***
- The indicative in-kind and financial contribution from industry beneficiaries is EUR 14 000 000 (target). ***NB: this amount is indicative and subject to change, pending approval by the IHI Governing Board.***

Due to the global nature of the participating industry partners, it is anticipated that some elements of the contributions will be in-kind contributions to operational activities (IKOP) from those countries that are neither part of the EU nor associated to the Horizon Europe programme.

The allocation of the EUR 200 000 financial contribution (FC) from industry beneficiaries will be decided by the full consortium at the second stage when preparing the full proposal.

The indicative in-kind contribution from industry beneficiaries may include in-kind contributions to additional activities (IKAA).

Indicative duration of the action

The indicative duration of the action is 60 months.

This duration is indicative only. At the second stage, the consortium selected at the first stage and the predefined industry consortium and contributing partner(s) may jointly agree on a different duration when submitting the full proposal. The expected timelines of key milestones to project kick-off are illustrated below:

Contribution of the pre-identified industry consortium

The pre-identified industry consortium expects to contribute to the IHI JU project by providing the following expertise and assets:

- Industry expertise in real world evidence, clinical development, benefit risk evaluation, regulatory affairs, HTA, health economics and market access for drugs, medical devices and drug/device combination products.
- Previously assessed and utilised use cases that can be utilised to evaluate existing methodologies, encountered challenges, explored pathways and practices for the use of RWD/E in healthcare decision making.

- Leverage synergies with existing initiatives, including H2O, EH DEN, IDEHRA, REDDIE, REALM, Real4Reg, EHR2EDC, GetReal Institute, TransCelerate, Duke Margolis Real World Evidence Collaborative, CIOMS, RWE4Decisions, and relevant EFPIA committees.⁵

Applicant consortium

The first stage applicant consortium is expected, in the short proposal, to address the scope and deliver on the expected outcomes of the topic, considering the expected contribution from the pre-identified industry consortium.

This may require mobilising the following expertise and/or resources:

- Comprehensive expertise in RWD & RWE including data science, standards & guidance.
- Expertise in the access, linkage and use of RWD and/or synthetic data to evaluate drugs, devices and drug/device combinations.
- Expertise in the technical, legal and ethical requirements to access and use patient data in Europe.
- Knowledge of drug and/or medical device development regulations,
- Expertise in interacting with regulatory authorities, national competent authorities, HTA bodies and payers.
- Experience with consumer-directed communications and/or patient advocacy (social media reach and expertise in health sector communications).
- Expertise in managing multi-stakeholder cross-sectoral projects.
- Citizens and/or patient representatives
- Real world data sources (healthcare providers, clinical sites, CROs, vendors, national/regional databases)
- Previous use cases that can be used evaluate existing methodologies, guidelines, and practices for the use of RWD/E in healthcare decision making.

The applicant consortium is expected to enable effective collaboration with regulatory authorities, national competent authorities, HTA bodies and payers, and may consider, for instance engaging them as consortium partners, or in an advisory capacity.

At the second stage, the public consortium selected at the first stage and the predefined industry consortium will form the full public-private consortium. The full consortium will develop the full proposal in partnership, including the overall structure of the work plan and the work packages, based upon the short proposal selected at the first stage.

Dissemination and exploitation obligations

The specific obligations described in the conditions of the calls and call management rules under 'Specific conditions on availability, accessibility and affordability' do not apply.

⁵ www.iderha.org, www.i-hd.eu/rd-and-collaborative-projects/ehr2edc, www.getreal-institute.org, www.transceleratebiopharmainc.com, <https://healthpolicy.duke.edu/projects/real-world-evidence-collaborative>, <https://cioms.ch/>