

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 1: A common European approach to equitable patient access to compassionate use

Expected outcomes

The action under this topic must contribute to all of the following outcomes:

1. An EU Compassionate Use (CU) framework for improved and more equitable patient access to CU across Europe.

This EU CU framework will include an overview of all existing CU pathways, best practices and recommendations for implementation. Healthcare systems and professionals, patients, regulators, industry and academia will benefit from this shared framework and implementation model increasing transparency and awareness, reducing fragmentation and variability between Member States and enabling improved and more equitable patient access to CU across Europe.

2. Greater consistency and efficiency in CU implementation.

Delivery of common governance and operational principles, streamlined terminology, processes and implementation tools for national competent authorities, healthcare systems, regulators, healthcare professionals and industry will enable common understanding, reduce administrative burdens, simplify processes and shorten lead-time to accessing CU.

3. Evidence generated on patients, health systems, and societal value.

Healthcare system stakeholders, policy and decision makers will gain evidence on patient outcomes, caregiver and physician burdens, access timelines, healthcare-system efficiency and impact on equity generated through research.

4. A fit-for-purpose “real-world data” collection framework for CU.

Regulators, health technology assessment (HTA) bodies/payers, researchers, industry, patients and healthcare professionals will benefit from a common data model, interoperable data standards and governance principles that improve the quality, usability and transparency of data collected through CU (real-world effectiveness and safety data). This will increase real-world evidence and enable more consistent assessment of patient, public health, and healthcare system impact.

5. Increased awareness, transparency, and stakeholder capability.

Educational resources, training programmes, implementation toolkits, transparency tools and communities of practice will be available and maintained beyond the project's end to improve all relevant stakeholders' awareness and capabilities to plan, access, implement and transition out of CU.

6. Sustainable European collaboration and governance beyond the project lifetime.

A sustainable European CU knowledge hub and a long-term governance model including measurable indicators to enable continued dissemination, uptake, exploitation and maintenance of

topic outputs and to inform future EU CU policy and practice will benefit the all relevant stakeholders.

Scope

Compassionate Use (CU), as defined under Article 83 of Regulation (EC) No 726/2004¹, is a regulatory pathway that enables physicians to request access to an unauthorised medicine for patients with a serious, life-threatening, or chronically debilitating disease who have no satisfactory treatment options available and cannot participate in a clinical trial. The primary purpose of CU is to provide treatment access for patients with a high unmet medical need. CU is distinct from both clinical research and commercial access: it is neither a clinical trial designed to generate evidence nor a reimbursement or market access programme. Depending on national regulations, CU may be provided from the pre-authorisation stage through to marketing authorisation and until the product becomes commercially available in the country of treatment.

Despite the existence of a European legal framework, implementation of CU remains highly fragmented across the EU Member States. Differences in national approaches, operational requirements, awareness, access routes, data collection practices, health-system readiness and timelines create inequities of access for patients dependent on where they live in Europe². Regulatory authorities operate within heterogeneous national frameworks, limiting opportunities for alignment. Industry faces substantial operational complexity through differing country requirements when providing multinational CU access. Healthcare professionals and patients lack awareness and understanding of the CU pathway. Researchers and policymakers lack access to the structured and comparable data needed to assess outcomes and inform future policy development. Transparency on the availability, implementation and outcomes of CU pathways remains limited.

Revisions to EU pharmaceutical legislation published in 2026 promote a common approach³. Article 27 suggests a common CU approach for both individuals and groups of patients. This is an opportunity to address the challenges in CU by establishing a more streamlined, consistent and evidence-based approach across Member States⁴.

The objective of this topic is to develop, pilot and evaluate a scalable European CU framework and implementation model that improves patient access, reduces inequities, strengthens operational efficiency, enhances stakeholder capabilities, increases transparency and establishes fit-for-purpose evidence-generation approaches. The action funded under this topic should create practical solutions and guidance that can be adopted by Member States, healthcare systems, regulators, healthcare professionals, patients, and industry across diverse national contexts.

Medicines, therapies (i.e. cell therapies, gene therapies) and medicines administered via a device (which may be novel) e.g. drug-device combination are within the scope of this topic.

The applicants are expected to undertake the following key research and innovation activities:

¹ [Regulation \(EC\) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency \(Text with EEA relevance\)](#)

² [Early access to medicines in Europe: Compassionate use to become a reality - EURORDIS-Rare Diseases Europe](#)

³ <https://data.consilium.europa.eu/doc/document/ST-7105-2026-INIT/en/pdf>

⁴ [An Industry Perspective on Compassionate Use in Europe: A Call for Change](#)

- Map current CU planning, implementation and patient transition approaches, barriers and facilitators, existence of sub-categories of CU and stakeholders' CU awareness and capabilities across Member States and industry;
- Identify best practices and shortcomings for healthcare professionals, patients, industry and health systems in providing, accessing and transitioning out of CU;
- Assess and determine how CU data can support understanding of the benefit–risk profile, supplement clinical trial data and inform regulatory, HTA body/payer, clinical and policy decision-making, and which CU data is best suited for this;
- Design and validate a fit-for-purpose CU 'RWD' framework, including common data elements, interoperability standards and governance principles. Ensure alignment with the evolving European Health Data Space (EHDS) and consider potential use for marketing authorisation applications and the new joint clinical assessment (JCA) process⁵;
- Conduct research to generate and disseminate evidence on patient outcomes, access equity, caregiver and physician burden, healthcare resource utilisation and societal value associated with CU;
- Develop a European CU framework and recommendations to inform and enable Member States and their healthcare system stakeholders to implement Article 27. The framework will incorporate common governance principles, streamlined terminology, operational steps/processes including templates e.g. for treatment guidance/standardised protocol, stakeholder responsibilities and risk-sharing/mitigation considerations;
- Conduct multi-country pilot studies and implementation research of proposed frameworks, recommendations and resources to assess feasibility, health-system impact and scalability;
- Develop methodologies and measures/indicators to assess healthcare system readiness for adoption of the proposed frameworks;
- Develop adoption approaches to ensure the uptake of the frameworks and recommendations supported by resources such as implementation tools, transparency tools and educational and training programmes/materials; these should be targeted to the relevant stakeholders including patients, patient organisations, healthcare professionals, regulators, HTA bodies/payers, Member States, academia, industry and health-system stakeholders;
- Consider and address ethical, legal and societal considerations along the topic including informed consent, transparency, equity, constraints to providing access;
- Ensure responsible use of data, digital and artificial intelligence-enabled tools where proposed, including considering robustness, traceability and awareness of relevant EU Artificial Intelligence Act requirements;
- Develop a sustainability plan and measures and a long-term governance model including:

⁵ Joint Clinical Assessments - Public Health - European Commission

- an EU CU knowledge hub or equivalent mechanism to make relevant project outputs accessible to stakeholders;
- a community of practice or a similar mechanism to support continued learning and dissemination beyond the project lifetime;
- resources, tools and digital solutions ensuring continued dissemination, uptake, exploitation and maintenance of topic outputs and continued evidence to inform future EU CU policy and practice.

Given the potential relevance of the topic outputs for future regulatory decision-making and health technology assessment (HTA)/payer processes, proposals should include both a regulatory engagement and interaction strategy involving the European Medicines Agency (EMA), Heads of Medicines Agencies (HMA), and national competent authorities, as well as a HTA body/payer engagement strategy and interaction plan. These activities should ensure that the frameworks, standards, guides, and tools developed are aligned with regulatory and HTA/payer expectations, support feasibility and long-term sustainability, and, where relevant, facilitate integration into future European guidance, regulatory practice, assessment frameworks, or policy approaches, including through the use of regulatory or policy sandboxes.

Expected impacts

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

- **Greater convergence of CU approaches across Member States** contributing to reduced geographic disparities in access, increased predictability of pathways, and support for more timely patient access.
- **Reduced operational burden and improved stakeholder capability** for patients, healthcare professionals, industry, regulators and health systems through practical guides, resources and toolkits enabling awareness, understanding, adoption and implementation.
- **Greater health system readiness and sustainable integration of CU into healthcare systems** through viable implementation mechanisms and a sustainable European community of practice, facilitating also CU for future health emergencies.
- **Strengthened generation and use of “real-world data (RWD)” from CU** supporting evidence-informed decision-making by regulators, HTA bodies/payers, healthcare professionals, and industry.
- **Improved regulatory and policy decision-making** supporting implementation of EU pharmaceutical legislation Article 27 and informing European CU⁶ and national guidelines, contributing to a more coherent early-access ecosystem.
- **Strengthened European competitiveness and attractiveness for health innovation contributing to key European health initiatives and strategic priorities.** A more

⁶ [Guideline on Compassionate Use of Medicinal Products, Pursuant to Article 83 of Regulation \(EC\), No 726/2004: https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/guideline-compassionate-use-medicinal-products-pursuant-article-83-regulation-ec-no-7262004_en.pdf.](https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/guideline-compassionate-use-medicinal-products-pursuant-article-83-regulation-ec-no-7262004_en.pdf)

predictable and efficient CU environment enables industry and healthcare systems to facilitate access to innovative therapies and strengthen health data infrastructure.

The action will also contribute to a number of European policies/initiatives, which include: the European Health Union, the [Regulation on the European Health Data Space \(EHDS\)](#) and broader EU efforts to improve health-system resilience, innovation uptake, and patient-centred care across Europe.

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

The challenges surrounding CU in Europe are inherently cross-border, multidisciplinary, and involve a diverse range of stakeholder groups. They cannot be effectively addressed by individual organisations, sectors, or Member States acting alone.

An IHI JU action provides the unique public-private framework required to mobilise the expertise, resources, and networks necessary to develop sustainable European solutions. The participation of the pharmaceutical and biotechnology industries is essential as it brings practical experience in CU implementation, evidence generation, and in collaboration across healthcare systems. Equally important is the involvement of regulatory authorities, HTA bodies/payers, healthcare professionals, researchers, academic institutions, patient organisations, and health ICT partners. Together, these stakeholders ensure that the proposed frameworks, methodologies, and digital solutions are scientifically robust, operationally feasible, patient-centred, and aligned with both national and European requirements.

The scale and ambition of the proposed action extend beyond the capacity of individual countries. Achieving the expected outcomes requires access to diverse datasets, infrastructures, expertise, and healthcare environments across Europe. Only a large-scale public-private partnership can overcome existing fragmentation, generate sufficiently robust evidence, validate solutions across multiple healthcare systems, and secure broad stakeholder acceptance and uptake.

By leveraging complementary capabilities across sectors, an IHI JU action can establish harmonised European frameworks, evidence-generation approaches, implementation tools, and communities of practice that support equitable access, health-system readiness, and effective CU beyond the project lifetime. The action therefore directly contributes to IHI JU objectives by improving access to innovative healthcare, generating high-quality evidence, and strengthening collaboration across the European health research and innovation ecosystem.

Pre-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 with regard to 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 between EUR 6 500 000 to 8 000 000.
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 between EUR 6 000 000 to 7 000 000 (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 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 may jointly agree on a different duration when submitting the full proposal.

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:

- Expertise in the planning and design considerations of CU implementation across Europe;
- Expertise in CU initiation, conduct, maintenance, closure and patient transition out of CU;
- Knowledge of current industry practices, barriers, feasibility constraints and opportunities for improving CU implementation;
- Experience with data collection in CU including practical considerations around a fit-for-purpose “RWD” framework including industry burden, data quality and governance;
- Expertise to support development of implementation frameworks and appropriate readiness and sustainability measures/indicators, operational guides, implementation tools, transparency tools and training resources;
- Expertise in the design, planning, and execution of RWD/RWE collection and use for regulatory approval/reimbursement of medicines and therapies;
- Know-how and experience of HTA/payer appraisals for medicines and therapies in Europe;
- Expertise in regulatory and HTA bodies/payer engagement strategies;
- Contribution to dissemination, sustainability and uptake of project outputs through industry networks and relevant stakeholder platforms;

- Where feasible and appropriate, industry may contribute to activities related to aggregate experience, case studies, provide operational insights or data contributions from CU to support the development and evaluation of the developed solutions including framework. Any such contribution would need to respect applicable legal, ethical, confidentiality, data protection and privacy requirements.

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, taking into account the expected contribution from the pre-identified industry consortium.

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

Patient and healthcare professional expertise

- Patients/patient organisations with demonstrated experience in CU⁷ e.g. co-development of CU programmes.
- HCPs and clinical centres with first-hand experience initiating, implementing and managing CU programmes.

Regulatory, HTA and access-decision-making expertise

- Regulatory science experts, HTA bodies/payers, health economists, and experts in evidence appraisal and uncertainty management with hands-on experience in CU.
- Experience engaging regulatory authorities, national competent authorities, HTA bodies and payers to support adoption and decision-making.

Evidence generation, data science, health-system transformation expertise

- Expertise in designing, implementing and evaluating health-system solutions and implementation frameworks, including implementation science, real-world data/evidence generation, health services research, patient-centred research, health policy and bioethics.
- Data science, interoperability, governance and AI expertise, including common data models, data standards, EHDS-related considerations, responsible AI development and use, and EU AI Act compliance.

Implementation, adoption and sustainability expertise

- Proven experience developing, piloting and evaluating implementation frameworks, operational tools, training approaches and health-system readiness assessments.
- Significant experience in developing and implementing sustainability plans, measures and governance models ensuring continued dissemination, uptake, exploitation and maintenance of topic outputs.

⁷ <https://www.clinicaltherapeutics.com/article/S0149-2918%2824%2900061-4/fulltext>

Programme and stakeholder management expertise

- Demonstrated experience managing complex, multi-country, multi-stakeholder European public-private partnerships, including stakeholder engagement and collaborative structures.

Regulatory authority engagement

- The applicant consortium is expected to enable effective collaboration with regulatory authorities and national competent authorities, for example as consortium partners or in an advisory capacity.

At the second stage, the consortium selected at the first stage and the predefined industry consortium will form the full 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.

Glossary

Acronym	Meaning
AI	Artificial Intelligence
CU	Compassionate Use
EC	European Commission
EU	European Union
EHDS	European Health Data Space
EMA	European Medicines Agency
HCPs	Healthcare Professionals
HTA	health technology assessment
HMA	Heads of Medicines Agencies
IHI JU	Innovative Health Initiative Joint Undertaking
IKAA	in-kind contributions to additional activities
IKOP	in-kind contributions to operational activities
ICT	Information & Communication Technology
JCA	Joint Clinical Assessment
RWD	Real-World Data
RWE	Real-World Evidence