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[1] (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 which will increase transparency and awareness, reduce fragmentation and variability between Member States and enable 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 the 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

[1] The definition of compassionate use can be found under scope.

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measurable indicators, will enable the continued dissemination, uptake, exploitation and maintenance of topic outputs as well as informing future EU CU policy and practice for the benefit of all relevant stakeholders.

Scope

European legislation provides the legal basis for the definition of Compassionate Use (CU). The current legal basis for CU is defined under Article 83 of Regulation (EC) No 726/2004[2], as making a medicinal product belonging to the categories referred to in Article 3(1) and (2) available for compassionate reasons to a group of patients with a chronically or seriously debilitating disease or whose disease is considered to be life-threatening, and who cannot be treated satisfactorily by an authorised medicinal product. The current legislation requires that the medicinal product concerned must either be the subject of an application for a marketing authorisation in accordance with Article 6 of this Regulation or must be undergoing clinical trials without explicitly restricting those trials to the specific indication for which CU is sought.

The proposed revision of the EU pharmaceutical legislation defines CU in Article 27[3] and introduces changes, for example, requiring that the product must be undergoing clinical trials for the same proposed therapeutic indication or mentioning real-world data (RWD) in context of CU.

To reflect the evolving regulatory landscape and the diversity of CU access and practices across Europe, for the purpose of this topic CU is defined in a broader way.

In the context of this topic, CU is a regulatory pathway that enables physicians to request access to an unauthorised medicine for either a group or individual patients with serious, life-threatening, or chronically debilitating diseases 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 for a marketing authorisation application nor a reimbursement or market access programme. Depending on national regulations, CU may be provided for indications inside or outside of the development plan 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 Europe[4]. 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.

[2] 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)

[3] New European Pharmaceutical Legislation - Proposed Article 27 CU

[4] Early access to medicines in Europe: Compassionate use to become a reality - EURORDIS-Rare Diseases Europe

Revisions to EU pharmaceutical legislation published in 2026 promote a common approach. This is an opportunity to address the challenges in CU by establishing a more streamlined, consistent and evidence-based approach across Member States[5].

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:

- 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 / 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) process[6];
- 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;

[5] An Industry Perspective on Compassionate Use in Europe: A Call for Change

[6] Joint clinical assessments for medicinal products - Public Health

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- 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[7] requirements;
- Develop a sustainability plan and measures and a long-term governance model including:
    - 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 CU[8] and national guidelines, **contributing to a more coherent early-access ecosystem.**

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[7] Regulation (EU) 2024/1689 of the European Parliament and of the Council of 13 June 2024 laying down harmonised rules on artificial intelligence and amending Regulations (EC) No 300/2008, (EU) No 167/2013, (EU) No 168/2013, (EU) 2018/858, (EU) 2018/1139 and (EU) 2019/2144 and Directives 2014/90/EU, (EU) 2016/797 and (EU) 2020/1828 (Artificial Intelligence Act)

[8] Guideline on Compassionate use of Medicinal Products

- **Strengthened European competitiveness and attractiveness for health innovation contributing to key European health initiatives and strategic priorities.** A more 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)[9] 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 collaboration across healthcare systems. Equally important is the involvement of regulatory authorities, HTA bodies / payers, healthcare professionals, researchers, academic institutions, patient organisations, and health information and communication technologies 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

The pre-identified industry consortium that will contribute to this cross-sectoral IHI JU project is composed of the following pharmaceutical and medical technology industry beneficiaries (‘constituent or affiliated entities of private members’):

- Bristol Myers Squibb
- Daiichi Sankyo
- Eli Lilly

[9] Regulation (EU) 2025/327 of the European Parliament and of the Council of 11 February 2025 on the European Health Data Space and amending Directive 2011/24/EU and Regulation (EU) 2024/2847

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- Gilead
- GSK
- Ipsen
- Merck KGaA
- Novartis (Lead)
- Roche
- Sanofi
- Servier
- UCB

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 up to EUR 7 000 000.
- The indicative in-kind and financial contribution from industry beneficiaries is EUR 6 180 000 (target).

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 pre-identified predefined industry consortium may jointly agree on a different duration when submitting the full proposal.

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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 the 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;
- Contribution to activities related to aggregate experience, case studies, operational insights or data contributions from CU to support proposed solutions, while respecting the applicable legal, ethical, confidentiality, data protection and privacy requirements. For example:
    i. qualitative multi-country case insights addressing cross-border operational navigation, local supply-chain constraints, and high-level administrative procedures;
    ii. historical numbers of requests by programme and country to benchmark access demand against disease prevalence and identify geographic inequities;
    iii. timeline data across key access intervals (request receipt, medical evaluation, product shipment, and first patient exposure) to analyse differences in timelines across European healthcare systems;
    iv. available data to support harmonised data extraction and robustness exercises.

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.

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

Patient and healthcare professional expertise

- Patients/patient organisations with demonstrated experience in CU[10] e.g. co-development of CU programmes.
- Healthcare professionals and clinical centres with first-hand experience initiating, implementing and managing CU programmes.

Regulatory, HTA and access-decision-making expertise

- Regulatory science experts, HTA expertise , 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.

Programme and stakeholder management expertise

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

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. Furthermore the applicant consortium is expected to include patients / patient organisations.

At the second stage, the applicant consortium selected at the first stage and the pre-identified 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.

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[10] https://www.clinicaltherapeutics.com/article/S0149-2918%2824%2900061-4/fulltext

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

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