CIRS Centre for Innovation in Regulatory Science

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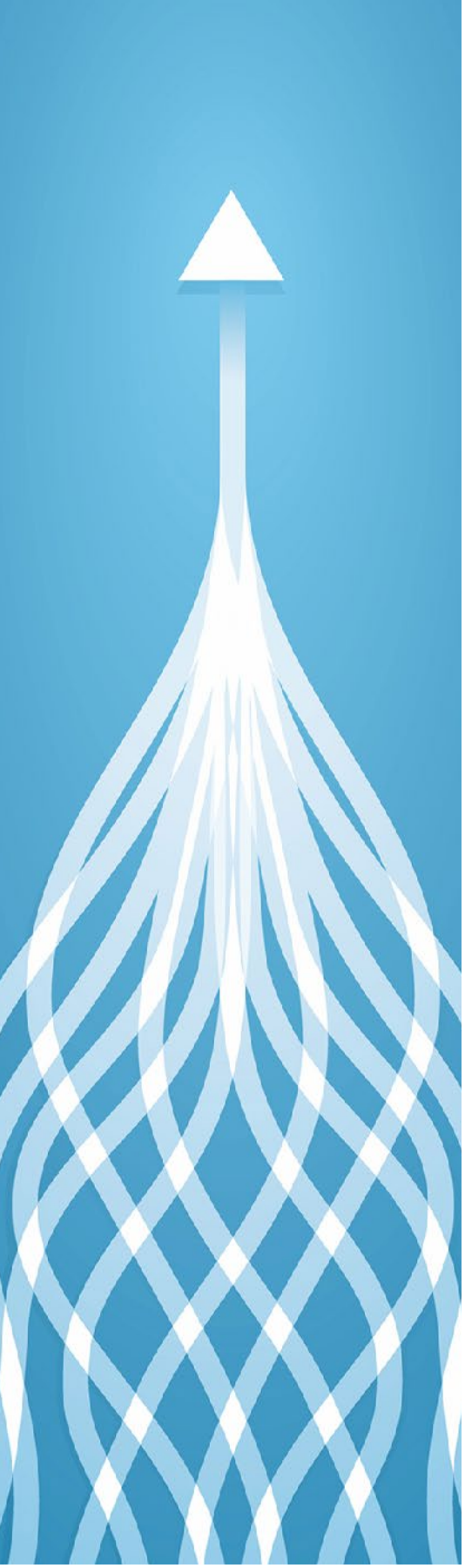
CIRS 2025 Research Agenda

Advancing regulatory and HTA policies and processes



CIRS Centre for Innovation in Regulatory Science

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Introduction and Overview

Who We Are

The Centre for Innovation in Regulatory Science (CIRS) is a neutral research organisation that provides a forum for policy leaders from government regulators, health technology assessment (HTA) agencies, the pharmaceutical industry, and other stakeholders in healthcare, such as patient organisations and academia.

CIRS focuses on improvements in policies and processes for regulation and HTA. We also support the development of agency capacity, including in low- and middle-income countries.

The CIRS team works internationally and is headquartered in the UK. CIRS works collaboratively with stakeholders worldwide, runs research projects internationally and conducts meetings globally to feed into and build on this research. Organisationally, CIRS is a wholly owned and independently managed UK subsidiary of Clarivate, with our funding derived from membership dues, special projects and grants from non-profits and governments.

CIRS's unique value proposition is its diverse community, with the participation of leaders from both small and large organisations in industry, regulators and HTA agencies around the world.

Three Pillars of CIRS Activities

For more information, please see 'About CIRS'.

Research Agenda 2024-2026

Executive Summary

CIRS generated its 2024-2026 research agenda with significant input from its Scientific Advisory Council and HTA Steering Committee. Priorities were identified and thoroughly reviewed through meetings of Topic Groups in 2022-2023, which focused on patient engagement; expedited pathways in regulation and HTA; and metrics.

Feedback from these groups, careful review of the landscape as well as conversations with various CIRS member companies, agencies and other stakeholders, were reviewed and organised into the following research agenda. This will be achieved through research projects, workshops and other meetings.

CIRS Research Agenda 2024 - 2026

Pillars	**Metrics** Evidence-driven insights into company and agency performance		
	Quality Improving decision-making processes		
	Alignment Converging stakeholder priorities and processes to accelerate patient access		
Themes	Decision-making framework for new ways of working and evidence generation technologies Moving implementation from concept to practicality	Good practices for regulatory and HTA collaborative models of review and assessment Sharing learnings and experiences	Metrics on new ways of working and evidence generation and their impact on decision making Strategic insights and impact measures
New Topics	Vaccines | Artificial Intelligence | Patient Engagement | High Impact Chronic Diseases | Rare Diseases		

Research Agenda 2024-2026

Themes

Decision-making frameworks for new ways of working and evidence generation techniques: Moving implementation from concept to practicality

Faced with increasingly complex technologies and novel evidence generation techniques, regulatory and HTA agencies are being challenged to work in new ways. There is pressure on them to be agile and effective in their processes and more efficient with their resources. While risk-based decision making and regulatory reliance are well-developed concepts for agencies, how to implement these in practice is not always clear.

This research area builds on previous CIRS work in the areas of advanced therapy medicinal products (ATMPs), digital health technologies, real-world data/evidence (RWD/E), risk-based decision making (reliance models) and the use of public and non-public assessment documentation. The overall aim is to shift regulatory and HTA frameworks and models in these areas from concept to practicality, so companies and agencies are supported to better implement these models.

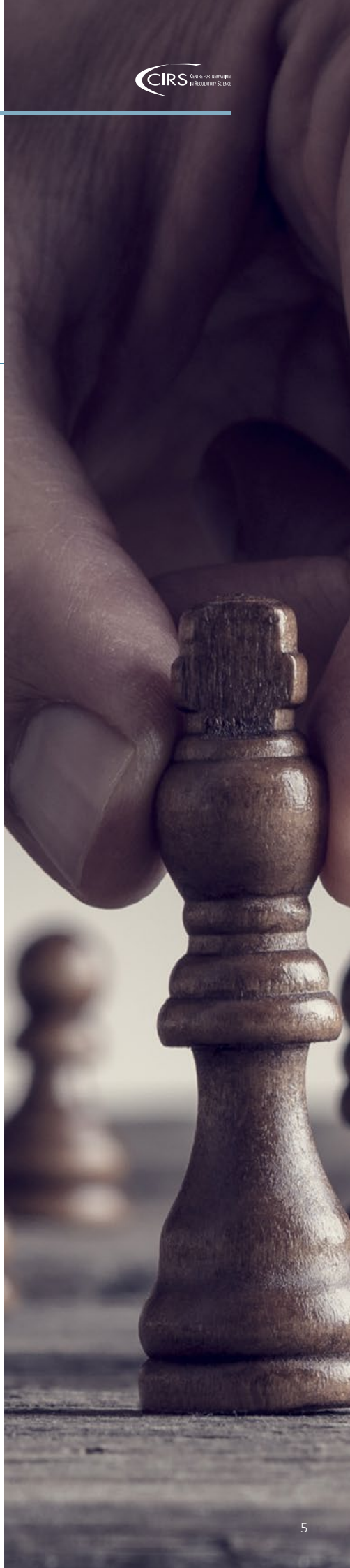
Good practices for regulatory and HTA collaborative models of review and assessment: Sharing learnings and experiences

More and more regulatory and HTA agencies are collaborating at a national, regional and/or international level as well as across disciplines. The impact of these collaborations, however, is still largely unknown. In particular there is uncertainty around the operationalisation of the EU HTA Regulation, which from 2025 will formalise collaboration between EMA and HTA bodies and collaboration between individual EU HTA bodies through Joint Scientific Consultation (JSC) and Joint Clinical Assessment (JCA). The assessment and funding environment for health products is also changing in other countries, for example with the passage of the US Inflation Reduction Act.

During 2024-2026, CIRS will assess the impact of regulatory and HTA collaborative models in both mature and growth markets, in order to share learnings and identify best practices. Collaborative models of interest are those that bring together regulators, HTA agencies, and both regulators and HTA agencies. Examples include Project Orbis, the Access Consortium, regional regulatory collaborations, the UK Innovative Licensing and Access Pathway (ILAP) and the EU JSC.

Metrics on new ways of working and evidence generation and their impact on decision making: Strategic insights and impact measures

CIRS' experience in benchmarking metrics goes back two decades. This research area continues and extends that solid foundation, with a focus for 2024-2026 to identify qualitative and quantitative metrics on the efficiency and effectiveness of regulatory review and HTA assessment. This includes assessing the impact of new ways of working, including novel methods of evidence generation and digital health technologies; the impact of legislative changes such as the revised EU General Pharma Legislation, EU HTA Regulation and US Inflation Reduction Act.



New research topics:

Patient engagement in regulatory and HTA decisions

Patients are increasingly involved in regulatory and HTA assessments, however, the impact that their input has on regulatory and HTA decision making is not well defined. During 2024-2026, CIRS will undertake stakeholder surveys, with a workshop dedicated to further understand the impact of patient engagement on regulatory and HTA agency decision making, including how this is measured and the needs, challenges and opportunities for patients as well as for companies and agencies going forward.

Vaccines

Interest in vaccines is growing from a public health, commercial, regulatory and HTA perspective, in part due to the COVID-19 pandemic, advances in vaccine technologies and the development of a significant number of new vaccines for adult use. During 2024-2026 CIRS will conduct a multi-stakeholder workshop to discuss the evolving vaccine landscape and how this can be better supported. In addition, we are looking to evaluate our metrics databases to identify metrics to collect as part of a potential research programme in this area.

High public health impact medicines for chronic diseases

Chronic diseases such as cardiovascular, metabolic and neurological diseases, may drive life expectancy down in significant populations but there are limited new drug development incentives for these diseases. A focus for CIRS during 2024-2026 will be exploring how such medicines could be incentivised and what learnings from other areas can be harnessed. Analysis of constraints and potential incentives for development of high impact medicines for chronic disease will be assessed through a workshop.

Artificial intelligence

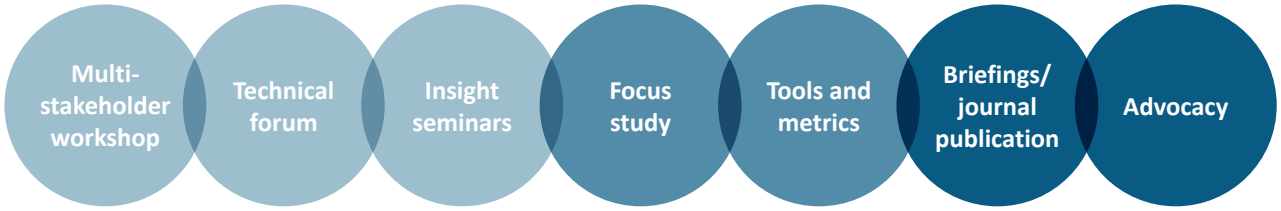
Artificial intelligence (AI) is being used by pharmaceutical companies, regulators and HTA agencies in a growing number of ways. During 2024-2026 CIRS will set up a Task Force to help design a stakeholder survey and AI Roundtable meeting that will inform future research areas for CIRS. These areas could address linkages that either exist or need to be strengthened between companies, regulators and HTA agencies for AI to be implemented efficiently but in a reliable, safe and effective manner.

Rare diseases

Rare diseases are increasingly seen as a large group of diseases that have common challenges and require a global response to accelerate drug development. During 2024-2026, CIRS will conduct metrics research on the regulatory and HTA landscape for rare diseases, including for the National Academies Committee on Regulatory Processes in the US and EU. From 2025, CIRS will participate in the Rare Diseases International-Lancet Commission on Rare Diseases (RDI-LCRD).

2025 Workstreams and Workshops

Outputs of the Regulatory and Access Programme (RaAP) span from multi-stakeholder workshops, to focus studies to advocacy activities:



CIRS members can participate in multi-stakeholder activities such as workshops, research and benchmarking metrics projects, benefit from priority access to CIRS publications and are part of an international community helping to shape major policy topics.

More information can be found in '**CIRS Membership**'.

The next sections outline the workplans for the CIRS Regulatory and HTA teams in 2025.

Global Development Workshop, February 2025

2025 Workstreams and Workshops

Regulatory Workstream for 2025

GOAL

Provide a neutral forum for the evolution of the global regulatory environment by facilitating the advancement of regulatory science concepts, tools and policies that improve the effectiveness, efficiency and decision making of companies and regulatory agencies in the development of safe and effective medicines.

PRIORITIES

TIME

Increase transparency of regulatory performance through timely, relevant metrics.

QUALITY

Enable development and implementation of good regulatory practices.

RISK-BASED

Promote the use of risk-based reviews (reliance, worksharing) and facilitated pathways.

TRANSPARENCY

Increase information sharing and transparent documentation of decision making

NEW MODELS

Evaluate new ways of working and support opportunities for alignment

2025 Workstreams and Workshops

Regulatory 2025 Workstream

Priority area	Outputs
1. Time	• R&D Briefings with updated metrics on mature regulatory agency benchmarking including assessment of Project Orbis and Access Consortium collaborations • Exploratory analysis on regulatory performance in other major markets including China and Brazil • Growth and Emerging Markets Metrics (GEMM) programme reports, analysis tool and Industry Discussion Meeting • Study using statistical analysis on the GEMM dataset to assess the link between CPP submission timing and medicine rollout • Metrics reports for agencies participating in the Optimising Efficiencies in Regulatory Agencies (OpERA) Programme, as well as external publications • Forum for regulatory agencies on 'Advancing performance of regulatory systems and enabling continuous improvement within agencies'

2. Quality	• Evaluation of practices and processes within target OpERA agencies using CIRS tools to ensure quality of process, practices and decision making
	  – Comparison and analysis of performance of WHO Maturity Level 3 agencies using OpERA Country Reports
	  – Training and education for OPERA agencies and regional bodies on implementation of benefit-risk frameworks, good review practices and quality decision making

3. Risk-based	• Report from the Latin American Systems to Enable Reliance (LASER-2) project • Support implementation of reliance in selected countries through training on benefit-risk assessment • Study of review models, timelines and Good Review Practices for the African ECOWAS region • Publication on the economic impact of reliance review in South Africa • Case study on the implementation of reliance by a national regulatory authority
4. Transparency	• Agency survey and multi-stakeholder workshop on 'Meaningful patient involvement in regulatory and HTA decision making – What are current practices and what impact does this have on the final assessment?'
5. New models	• Multi-stakeholder Roundtable on 'AI – Optimising company-regulator-HTA agency interactions through the drug product lifecycle' • Multi-stakeholder workshop on 'High public health impact medicines for chronic diseases- Do regulatory, HTA and payer paradigms need to change?

+ Regulatory Technical Forum for CIRS member companies (Topic tbc)

2025 Workstreams and Workshops

HTA Workstream for 2025

GOAL

Provide a neutral forum for the evolution of the global HTA environment by advancing the process and policies that improve the effectiveness, efficiency and decision making of companies and HTA agencies in improving access to new medicines.

PRIORITIES

PERFORMANCE
Increase HTA performance and transparency through timely, relevant metrics.

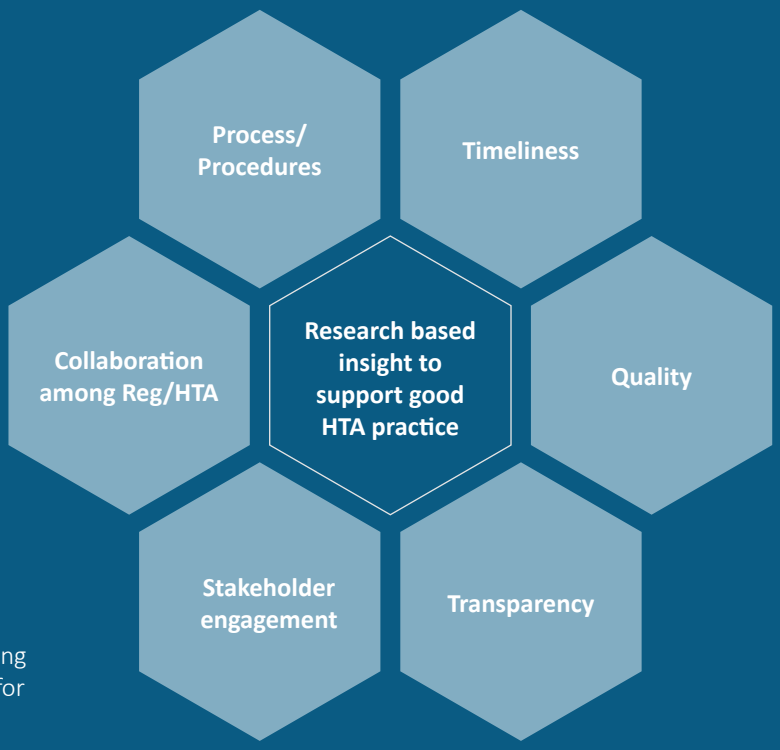
EARLY INSIGHT
Identify opportunities and challenges with early HTA advice and enhance development strategies and upstream communication, including with regulatory agencies

COLLABORATION
Promote international engagement and strategic policy discussion

QUALITY
Advance the development of good HTA practices

TRANSPARENCY
Increase information sharing and transparent documentation of decision making

NEW MODELS
Evaluate new ways of working and support opportunities for collaboration



2025 Workstreams and Workshops

HTA 2025 Workstream

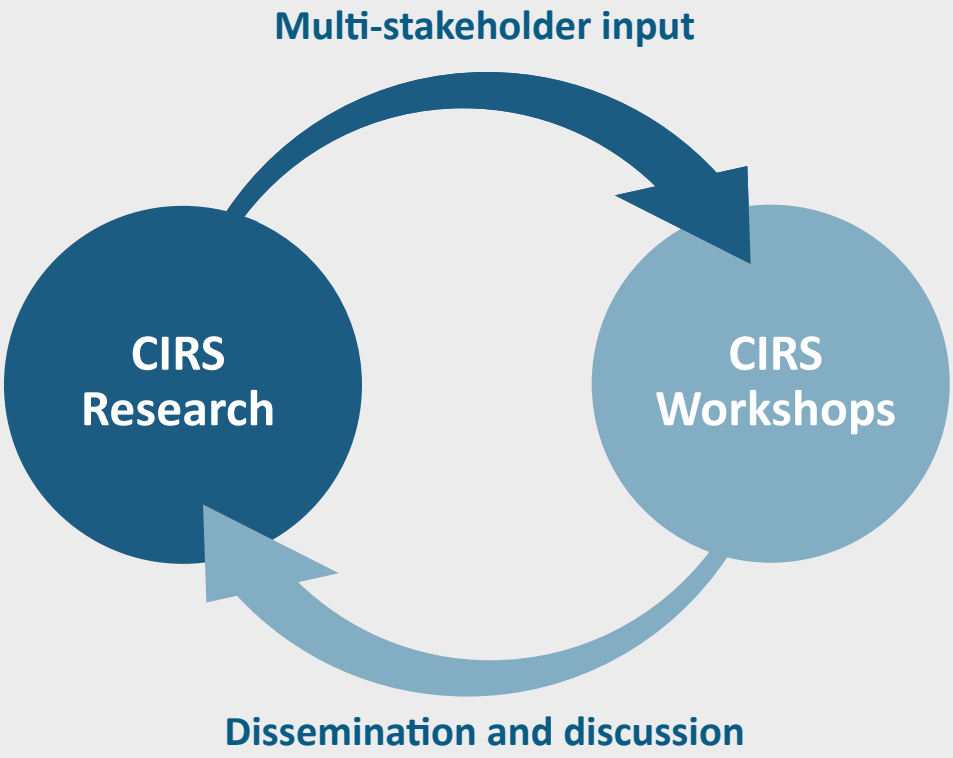
Priority area	Outputs
 1. Performance	• Metrics study: EMA oncology approvals (2018–2023) and HTA recommendations across six EU member states- baseline for assessing the impact of the EU HTA Regulation from 2025 • HTADock database and annual R&D Briefing following HTA timelines and outcomes of new active substances in 12 key jurisdictions (HTADock project)
 2. Early insight	• Metrics study: Early HTA Advice: Assessing Experiences, Addressing Capacity Challenges, and Improving Processes for Greater Impact • Publication on 'Enhancing development strategies through early scientific advice from HTA agencies – Experiences, expectations and best practices from health technology developers'
 3. Collaboration	• Publication on 'Ensuring the Efficiency and Effectiveness of Joint Clinical Assessment in National HTA Decision-Making: Insights from the 2024 CIRS multi-stakeholder workshop' • HTA focused multi-stakeholder workshop on 'Navigating national decision making post-Joint Clinical Assessment (JCA): Enablers, barriers and the path forward'
 4. Quality	• A literature review : The impact of patient involvement on Health Technology Assessments • Metrics study: Publication on HTA submission strategies and their associations with the time to HTA recommendation and the type of HTA recommendation in Australia and Canada
 5. Transparency	• Agency survey and multi-stakeholder workshop on 'Meaningful patient involvement in regulatory and HTA decision making – What are current practices and what impact does this have on the final assessment?'
 6. New models	• Multi-stakeholder Roundtable on 'AI – Optimising company-regulator-HTA agency interactions through the drug product lifecycle' • Multi-stakeholder workshop on 'High public health impact medicines for chronic diseases- Do regulatory, HTA and payer paradigms need to change?

+ HTA Technical Forum for CIRS member companies (Topic tbc)

2025 Workstreams and Workshops

2025 Workshops

CIRS workshops provide a forum for the dissemination and discussion of CIRS research through structured sessions and breakout groups that enable companies, agencies and other participants to shape CIRS research projects. A synopsis and report are published following each workshop to help disseminate the learnings and recommendations from the meeting.




Regulatory-HTA Alignment Workshop, October 2024

2025 Workshops

Regulatory agency collaboration and system strengthening – How is this enabling national, regional and continental models and improving medicines availability for patients?

26-27th February, Johannesburg, South Africa

Objectives:

- Identify the critical success factors and activities that agencies need to put in place to strengthen their regulatory systems for the registration of medicines and how this is being carried out at a jurisdictional (national) level and through regional work-sharing initiatives.
- Discuss lessons learned from the current initiatives and practices that enable jurisdictions and regional work-sharing initiatives to move from concept to practical implementation as well as the key challenges and opportunities.
- Make recommendations on how jurisdictional system strengthening and regulatory collaboration can drive continental models across agencies and improve medicines availability for patients globally.

High public health impact medicines for chronic diseases- Do regulatory, HTA and payer paradigms need to change?

12-13th June, Tysons Corner, Virginia, USA

Objectives:

- Review and discuss high public health impact medicines for common chronic diseases to understand the challenges these medicines face from a regulatory, HTA, payer and patient perspective.
- Identify how to incentivise medicines which target diseases of significant public health interest that drive life expectancy down.
- Propose options and make recommendations on how to address policy challenges in the development, regulation, HTA and funding of high public health impact medicines for common chronic diseases. This includes the particular policy challenges for the development and regulatory and HTA review of drugs that can modify or potentially reverse a disease, rather than simply treating the symptoms of disease.

2025 Workshops

Meaningful patient involvement in regulatory and HTA decision making – What are current practices and what impact does this have on the final assessment?

1st-2nd October, Slough, UK

Objectives:

- Discuss the value of engaging patients in early development and how this aids downstream decision making.
- Clarify how regulatory and HTA agencies are utilising patient engagement (PE) and patient experience data (PED) within their review and assessment frameworks.
- Identify the challenges and opportunities for measuring the utilisation of patient input in the evaluation of new medicines and how this can be best articulated in assessment reports.
- Make recommendations on what should be the key components of a systematic structured approach to documenting how PE/PED was used during the assessment and the articulation of its influence on agency decision making.

Navigating national decision making post-Joint Clinical Assessment (JCA): Enablers, barriers and the path forward

27th November, Amsterdam, The Netherlands

Objectives:

- Examine the role of HTA agencies in the JCA process – focusing on both process inputs and outputs, as well as how agencies and companies are adapting their processes to integrate JCA outputs into decision making.
- Identify key challenges and capacity building needs for effective JCA implementation, including resource constraints, methodological and timeline alignment, and stakeholder engagement.
- Facilitate multi-stakeholder discussions and develop recommendations, bringing together HTA agencies and industry to explore practical implementation of JCA outputs and identify actions to improve efficiency and alignment in national decision making.

More About CIRS and How We Work

Collaborations

CIRS has a rich history of collaboration with various groups on topics of mutual interest. In the near past and present, these have included:

- Gates Foundation
- National Academies of Sciences, Engineering, and Medicine
- Rare Diseases International-Lancet Commission on Rare Diseases
- International Coalition of Medicines Regulatory Authorities (ICMRA)
- World Health Organization (WHO)
- International Council for Harmonisation of Technical Requirements for Pharmaceuticals (ICH)
- United States Pharmacopoeia (USP)
- Office of Health Economics (OHE)
- African Union Development Agency- New Partnership for Africa's Development (AUDA-NEPAD)
- Centre of Regulatory Excellence, Singapore (CoRE)
- University of Hertfordshire
- Utrecht University
- University of Southern California.

Collaborations on Regulatory Strengthening

CIRS is currently working with 35+ regulatory authorities globally through the Optimising Efficiencies in Regulatory Agencies (OpERA) programme, which is supported by the Gates Foundation.

A key activity of the OpERA programme is to promote the use of CIRS tools that help regulators to implement WHO indicators and sub-indicators as part of the WHO Global Benchmarking Tool assessment programme. This ultimately helps regulators to become more effective, efficient, predictable, accountable, and high-performance based.

For more information about CIRS, please see 'About CIRS'.

CIRS Centre for Innovation in Regulatory Science

About CIRS

The Centre for Innovation in Regulatory Science (CIRS) is a neutral, independent UK-based subsidiary of Clarivate plc. Its mission is to identify and apply scientific principles for the purpose of advancing regulatory and health technology assessment (HTA) policies and processes. CIRS provides an international forum for industry, regulators, HTA bodies and other healthcare stakeholders to meet, debate and develop regulatory and reimbursement policy. It is governed and operated by Clarivate for the sole support of its members' activities. The organisation has its own dedicated management and advisory boards, and its funding is derived from membership dues, related activities, and grants.

Contact Us

Anna Somuyiwa, Head of CIRS	asomuyiwa@cirsci.org
Dr Mario Alanis, Senior Consultant	marioalanisgarza@cirsci.org
Dr Magda Bujar, Associate Director, Regulatory Programme and Strategic Partnerships	mbujar@cirsci.org
Penelope Cervelo Bouzo, Research Analyst	pcervelo@cirsci.org
Gill Hepton, Administrator	ghepton@cirsci.org
Adem Kermad, Principal Research Analyst	akermad@cirsci.org
Juan Lara, Senior Research Analyst	jlara@cirsci.org
Dr Neil McAuslane, Scientific Director	nmcauslane@cirsci.org
Dr Jenny Sharpe, Communications Manager	jsharpe@cirsci.org
Dr Belén Sola, Senior Research Analyst	bsola@cirsci.org
Prof Stuart Walker, Founder and Senior Advisor	swalker@cirsci.org
Dr Tina Wang, Associate Director, HTA Programme and Strategic Partnerships	twang@cirsci.org

Centre for Innovation in Regulatory Science (CIRS)

70 St Mary Axe, London EC3A 8BE, UK

cirs@cirsci.org

www.cirsci.org

www.linkedin.com/company/centre-for-innovation-in-regulatory-science-ltd/

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