CIRS Centre for Innovation in Regulatory Science

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Aligning global value-based decision making

CIRS 2024 Research Agenda

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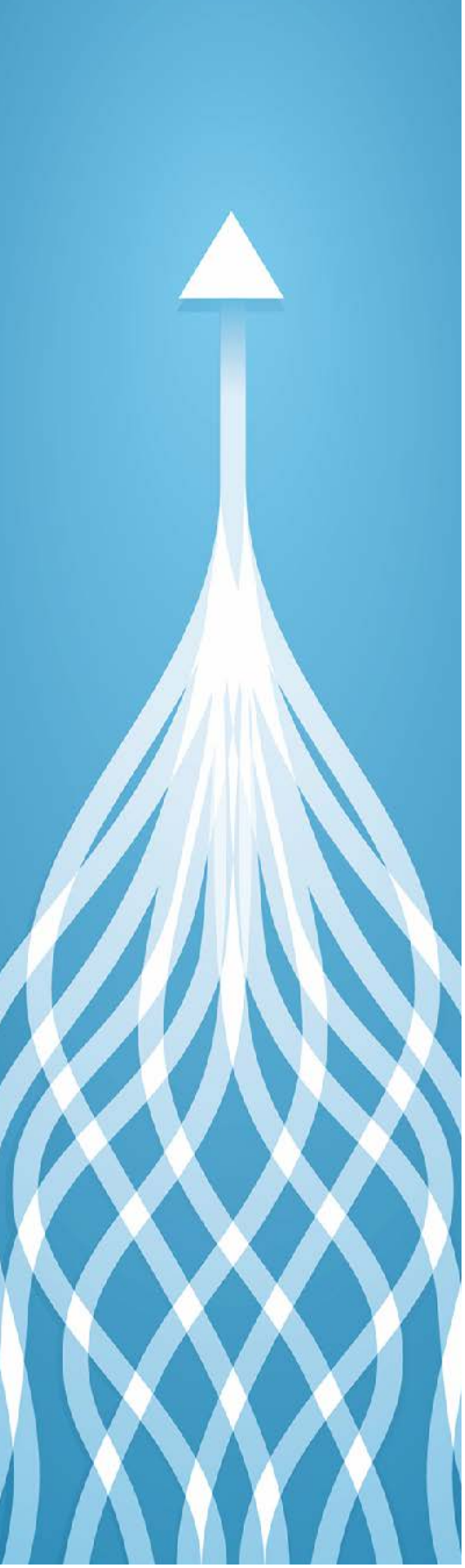

CIRS Centre for Innovation in Regulatory Science

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Introduction and overview

Who we are

The Centre for Innovation in Regulatory Science (CIRS) is a neutral, scientific and independently run global 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.

We serve patients, agencies and medical developers by focusing on improvements in policies and processes for regulation and HTA. CIRS also supports 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 priorities 2024-2026

Executive summary

CIRS generated its 2024-2026 research plan with significant input from its Scientific Advisory Council and HTA Steering Committee. Priority areas were identified and fleshed out through meetings of Topic Groups in 2022-2023, which focused on:

- **Patient engagement**
- **Expedited pathways in regulation and HTA**
- **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 strategy. This will be achieved through research projects, workshops and other meetings, and is grouped into three themes:

Enabling decision-making frameworks for new ways of working and evidence generation technologies -

Moving implementation from concept to practicality

Defining and promoting good practices for regulatory and HTA collaborative models of review and assessment

Metrics on new ways of working and evidence generation and their impact on decision making

Strategic insights and impact measures

NEW FOCUS: VACCINES, PATIENT ENGAGEMENT, HIGH IMPACT CHRONIC DISEASES

Research priorities 2024-2026

Focus areas

Enabling 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.

Defining and promoting good practices for regulatory and HTA collaborative models of review and assessment

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.

These new areas of focus will be incorporated into the three overarching research priorities:

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

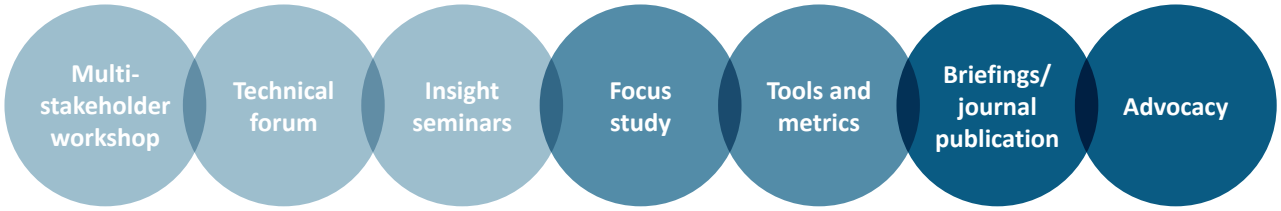
Interest in vaccines is growing from a public health, commercial, regulatory and HTA perspective, in part due to the COVID-19 pandemic, advances in vaccine technologies and the development of a significant number of new vaccines for adult use. During 2024-2026 CIRS will conduct a workshop to discuss the evolving vaccine landscape and how this can be better supported for the future. 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 initially introduced as part of existing topics and workshops during this research cycle, with consideration of a dedicated workshop within the cycle.

Focus on 2024 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 2024.

Focus on 2024 workstreams and workshops

Regulatory workstream for 2024

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

Focus on 2024 workstreams and workshops

Regulatory 2024 Workplan

Priority area	Outputs
1. Time	• R&D Briefing with updated metrics on mature regulatory agency benchmarking – also scoping for additional jurisdictions • Growth and Emerging Markets Metrics (GEMM) programme reports and online analysis tool • Metrics reports from agencies participating in the Optimising Efficiencies in Regulatory Agencies (OpERA) Programme • Supporting the National Academy of Sciences, Engineering and Medicines on a study on processes for evaluating the safety and efficacy of drugs for rare diseases or conditions in the US and the EU
2. Quality	• Evaluation of practices and processes within target OpERA agencies – publication of country reports • Studies on implementation of benefit risk frameworks, good review practices and quality decision making by OpERA agencies and regional bodies • Reports from a study on the implementation of Good Reliance Practices (GRelP) by the Andean national regulatory authorities • Report from study assessing implementation and adherence to International Council for Harmonisation of Technical Requirements for Pharmaceuticals (ICH) Guidelines
3. Risk-based	• R&D Briefing on approaches to implementing reliance including considerations for agencies • Regulators' Forum on 'Ensuring timely availability of medicines through regulatory collaboration – how can agencies build trust and work together to ensure a fit-for-purpose toolbox?' • Report from the Latin American Systems to Enable Reliance (LASER-2) project
4. Transparency	• R&D Briefing on a study appraising public assessment reports as tools for reliance • R&D Briefing on a study evaluating industry and agency experiences on the use of assessment reports
5. New models	• Publication from a study focused on the development of an artificial intelligence tool to aid in the evaluation of quality issues in the risk-based assessment of new medicines • Multi-stakeholder workshop on 'New ways of working – Enabling patient access through reliance or regional review models' • Multi-stakeholder workshop on 'Vaccines – Are regulatory and funding approaches fit for purpose for the next decade?' • Multi-stakeholder workshop on 'New ways of working across regulatory and HTA agencies: collaborative, work-sharing or reliance models- What are the policy implications for companies, HTA and regulatory agencies?' • Industry Technical Forum

Focus on 2024 workstreams and workshops

HTA workstream

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.

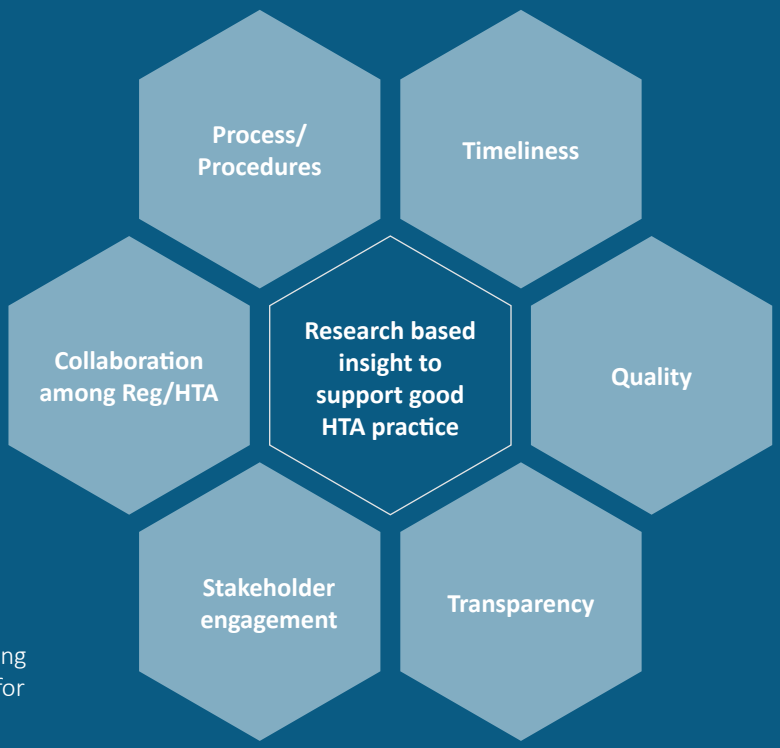
EARLY INSIGHT
Identify opportunities and challenges with early HTA advice and enhance development strategies and upstream communication, including with regulatory agencies

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



Focus on 2024 workstreams and workshops

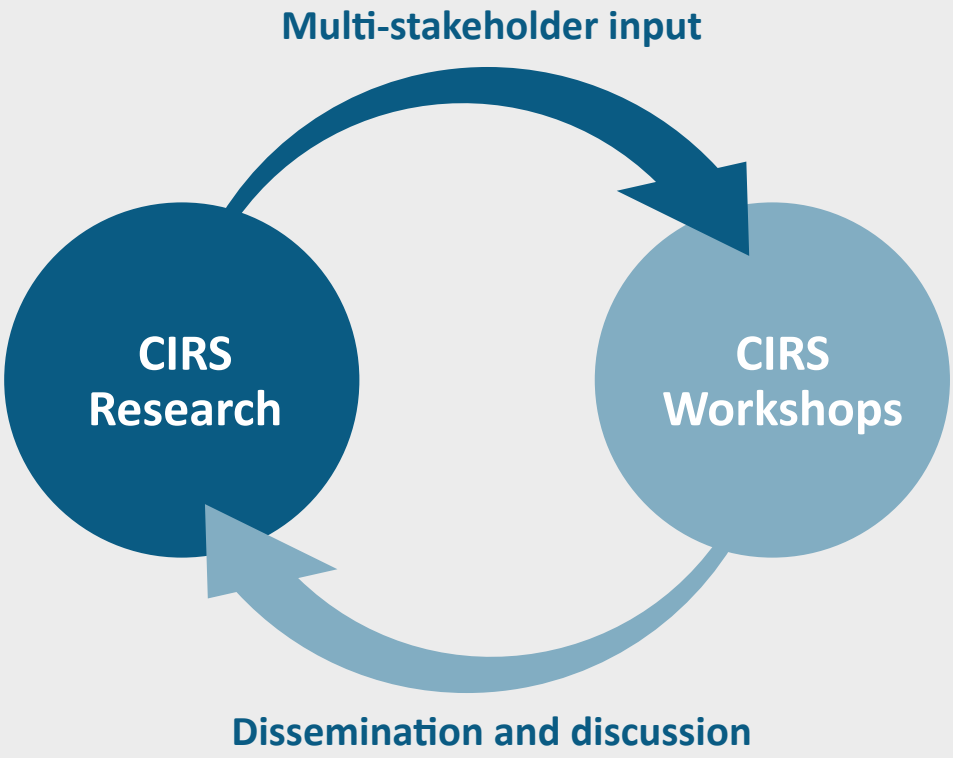
HTA 2024 Workplan

Priority area	Outputs
 1. Performance	• HTADock Database and Briefing follows HTA timelines and outcomes of new active substances in 9 key jurisdictions (HTADock project). • 2024 HTADock Database expanded to additional HTA agencies in the EU to set the baseline to monitor drug roll out prior to the EU HTA Regulation.
 2. Early insight	• The HTA Industry Metrics Programme has evolved to focus on early HTA advice; individual products are being tracked to assess implementation, experiences and utilisation of early HTA advice and early parallel advice by regulator and HTA agencies.
 3. Collaboration	• Multi-stakeholder workshop on 'Facilitating Joint Clinical Assessment (JCA) Implementation, Utilisation and Timely Patient Access: Considerations and measures to assess efficiency and effectiveness of the process to enable iterative learnings among stakeholders.' • Multi-stakeholder workshop on 'New ways of working across regulatory and HTA agencies: collaborative, work-sharing or reliance models- What are the policy implications for companies, HTA and regulatory agencies?'
 4. Quality	• Research manuscript developed and submitted investigating the regulatory and HTA submission strategy in terms of parallel and sequential submission and influencing factors for the review sequence. • An industry-focused survey and Technical Forum to investigate the evolving role of HTA policy company professionals, including the challenges, opportunities and cross-cutting learnings between the groups and across companies on best practices.
 5. Transparency	• An R&D Briefing to assess the common products that were approved and assessed in Australia, Canada and the UK in terms of roll out strategy of new medicines and the impact of the Access Consortium and Project Orbis. • An R&D Briefing focusing on the divergencies and similarities in decision making of 12 EU HTA agencies, by collecting data from public assessment reports/recommendations.
 6. New models	• An industry-focused survey aimed at evaluating companies' preparedness for the EU HTA Regulation, focusing on internal structure, expertise, processes and strategies. • An R&D Briefing on the utilisation of digital health technology for evidence generation to improve regulatory/HTA decision making. • Multi-stakeholder workshop on 'Vaccines – Are regulatory and HTA approaches fit for purpose for the next decade?'

Focus on 2024 workstreams and workshops

2024 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 report is published following each workshop to help disseminate the learnings and recommendations from the meeting.



2024 Workshops

New ways of working- Enabling patient access through reliance or regional review models

28-29th February 2024, Sao Paulo, Brazil

Objectives:

- Identify current risk-based prioritisation evaluation models of decision making being used for the review of medicines, and the benefits and hurdles of utilising these in the review of new medicines.
- Discuss frameworks and decision-making practices that need to be in place to move from concept to practical implementation for both unilateral and regional reliance models.
- Make recommendations on practical considerations and current best practices for both unilateral and regional models of reliance, discussing what is necessary to allow agencies to focus on value-added activities in order to provide timely patient availability to good quality, safe and effective medicines.

Vaccines – Are regulatory and funding approaches fit for purpose for the next decade?

13-14th June 2024, Tysons Corner, Virginia, USA

Objectives:

- Review and discuss the changing vaccine landscape and what the opportunities and challenges are within and across development, regulatory and HTA.
- Identify critical information gaps and how regulatory and HTA systems need to evolve to accommodate new vaccine technologies.
- Propose options and make recommendations on how to address policy challenges in the development, regulation, HTA and funding for vaccines.

2024 Workshops

Facilitating Joint Clinical Assessment (JCA) implementation, utilisation and timely patient access: Considerations and measures to assess efficiency and effectiveness of the process to enable iterative learnings among stakeholders

14th June 2024, Barcelo Sevilla Renacimiento, Seville, Spain

Objectives:

- Identify current process and procedures of HTA agencies in EU Member States, and companies' local submission approach: What needs to be changed or in place to move from concept to practical implementation of JCA?
- Discuss critical challenges and potential solutions for implementing JCA in the national decision-making process: What is necessary to allow agencies to focus on value-added activities in order to provide timely patient availability building on the JCA?
- Make recommendations on assessing the efficiency and effectiveness of JCA: What research is required to assess the short- and long-term goals of JCA? What indicators are required to enable iterative learning among stakeholders?

New ways of working across regulatory and HTA agencies: collaborative, work-sharing or reliance models- What are the policy implications for companies, HTA and regulatory agencies?

8-9th October, UK

Objectives:

- Assess the impact of different regulatory and HTA collaborative models on development, regulatory review and HTA assessment.
- Understand the experiences and learnings from current regulatory/regulatory, HTA/HTA and regulatory/HTA collaborative models- what can be learnt at local, regional, national, and international levels? How do these models influence companies' development strategy and jurisdictional roll out?
- Make recommendations on the current and future development of regulatory and HTA collaboration, such as the EU HTA Regulation and its jurisdictional implementation, international initiatives outside of Europe and cross-continent partnerships.

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:

- Bill and Melinda Gates Foundation (BMGF)
- 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.

In 2024, we look forward to participating in conferences held by the Drug Information Association (DIA), HTA International (HTAi), The Professional Society for Health Economics and Outcomes Research (ISPOR), The Organisation for Professionals in Regulatory Affairs (TOPRA) and the Regulatory Affairs Professionals Society (RAPS).

Collaborations on Regulatory Strengthening

CIRS is currently working with 35+ regulatory authorities globally through the Optimising Efficiencies in Regulatory Agencies (OpERA) programme, which is in part supported by grants from BMGF and USP.

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 maintain a leadership role in identifying and applying 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 through the innovative application of regulatory science. 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.

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