Review of 2024

Key research outputs and meetings

In 2024 CIRS continued to deliver for its stakeholders

4

Journal publications

6

CIRS R&D Briefings

4.7

Out of 5 feedback score for multi-stakeholder workshops

19

Conference presentations

764

New LinkedIn followers

Regulatory and HTA Research Programmes

2024 Regulatory Research Outputs

Time

- R&D Briefing with updated metrics on mature regulatory agency benchmarking
- 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
- 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

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

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
- 3 peer-reviewed publications and a PhD thesis on regional collaborative models in Africa

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
- Webinar sharing results of above studies

New Models

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

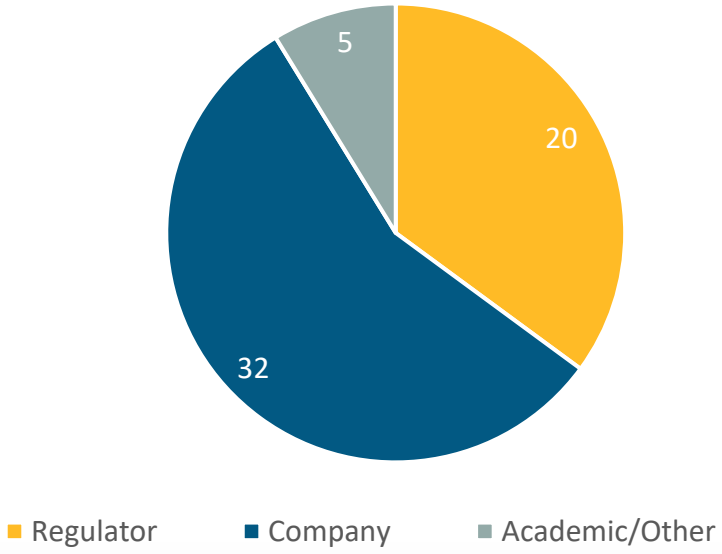
2024 HTA Research Outputs

Performance	2024 HTADock Database and Briefing follows HTA timelines and outcomes of new active substances in 9 key jurisdictions with the potential to expand to further EU member states.
Early insight	CIRS HTA strategic forum: Evidence-Based Metrics - Supporting Good HTA Decision Making
Collaboration	Two multi-stakeholder workshops bringing HTA agencies and companies together to examine: • 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 (June 2024). • 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? (October 2024)
Quality	• Research manuscript: Ensuring the efficiency and effectiveness of Joint Clinical Assessment in national HTA decision making
Transparency	• 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. • Research to assess the frequency and variation of clock-stop during EMA Assessment for oncology products – implication on JCA timelines.
New Models	• An industry-focused survey aimed at evaluating companies' preparedness for the EU HTA Regulation, focusing on internal structure, expertise, processes, and strategies. • Industry Technical Forum – 'Meaningful patient involvement in regulatory and HTA decision making – What are current practices and what impact does this have on the final assessment?'

Workshops and Meetings

Workshop 28-29th Feb 2024 - New ways of working: Enabling patient access through reliance or regional review models

- Identify current risk-based prioritisation evaluation models of decision making being used for the review of medicines, 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.



65
attendees

18
countries

4.6/5
Feedback score

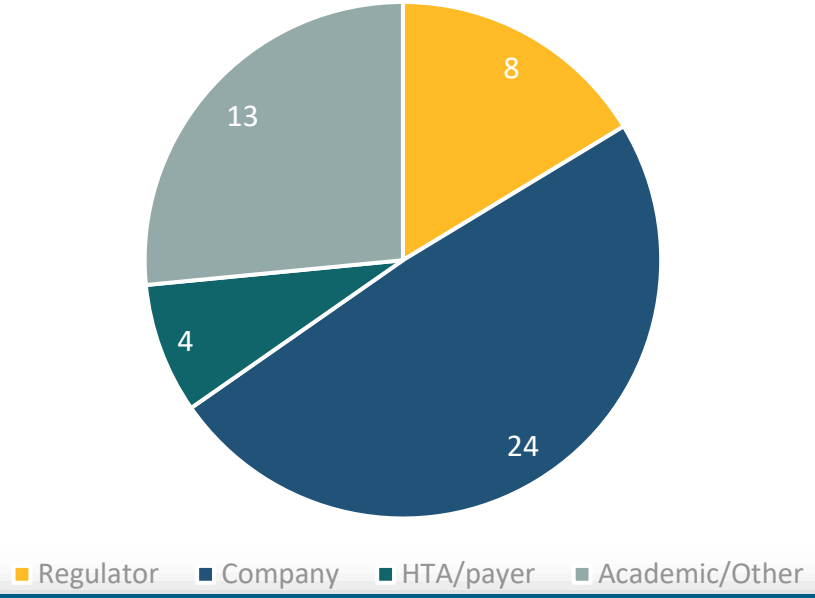
Agencies included:

- Regulatory: Brazil, Ecuador, Peru, Chile, Mexico, Cuba, El Salvador, Europe (EMA), Switzerland, Canada, South Africa, Malaysia, Egypt, Tanzania

LINK TO REPORTS

Workshop 13-14th June 2024 – Vaccines: Are regulatory and funding approaches fit for purpose for the next decade?

- Review and discuss the changing vaccine landscape and what the opportunities and challenges are within and across development, regulatory, HTA agencies and National Immunisation Technical Advisory Groups (NITAGs).
- Identify critical information gaps and how regulatory and HTA/NITAG 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.



49
attendees

13
countries

4.8/5
Feedback score

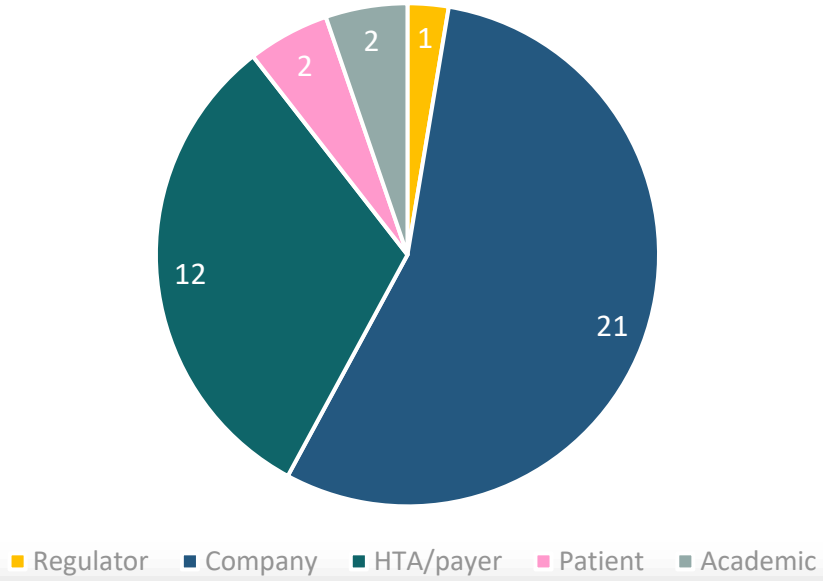
Stakeholders included:

- Regulatory: FDA, Health Canada, Swissmedic, ANVISA, HSA
- HTA/payers from: Canada, Austria, Spain, Germany
- CDC, NIAID, BARDA, PCORI

LINK TO REPORTS

Workshop 14th June 2024 – Facilitating joint clinical assessment (JCA) implementation, utilisation and timely patient access

- Identify current process and procedures of HTA agencies in Member States, and companies' local submission approach.
- Discuss critical challenges and potential solutions for implementing JCA in the national decision-making process.
- 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?



38
attendees

16
countries

4.7/5
Feedback score

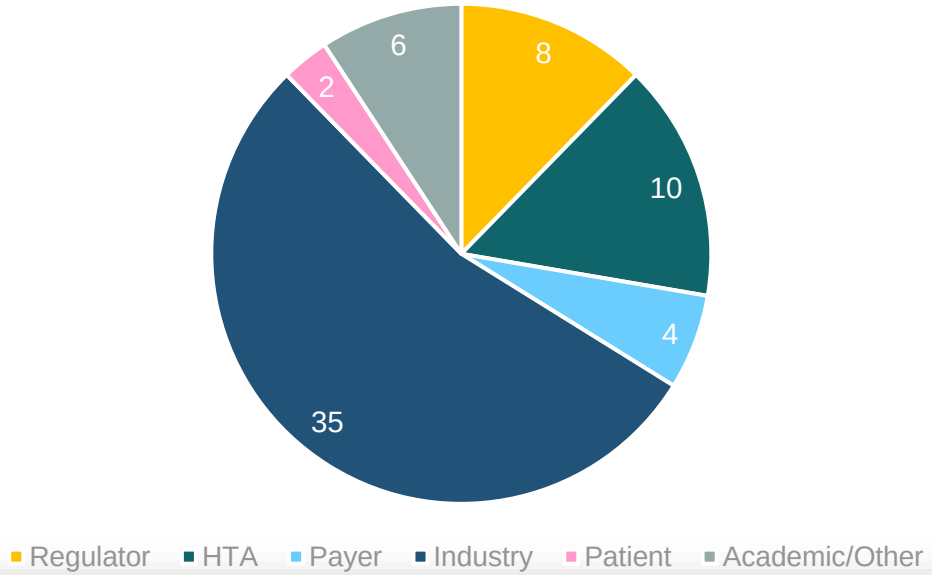
Stakeholders included:

- HTA agencies: Netherlands, Spain, Poland, Bulgaria, Romania, Norway, UK, Canada
- Payers from: Germany, Belgium
- EMA
- European Patients Forum

LINK TO REPORTS

Workshop 9-10th October 2024 – New ways of working across regulatory and HTA agencies: Collaborative, work-sharing and reliance models

- 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.
- 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 etc.



65
attendees

18
countries

4.6/5
Feedback score

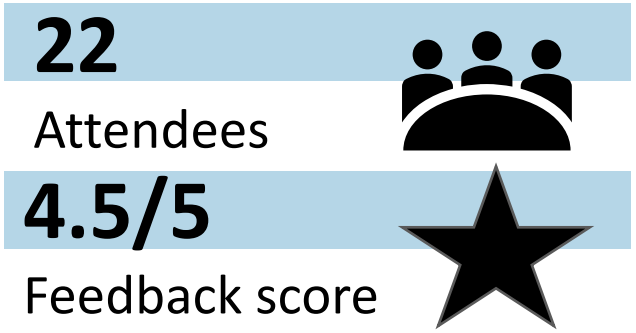
Stakeholders included:

- Reg: US FDA, EMA, MEB, Health Canada, Swissmedic
- HTA: NICE, TLV, CDA, SMC, CDE (Taiwan), HITAP
- Payers from: Austria, Germany, Belgium
- ICER, EURORDIS, Cancer Patients Europe

LINK TO SYNOPSIS

Regulatory and Access Programme Industry Technical Forum, 5th November 2024
Patient engagement and patient experience data: How is this supporting review and reimbursement decisions and can impact be measured?

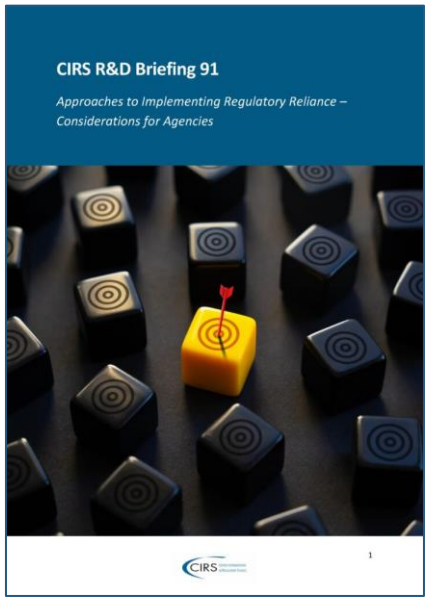
- Improve understanding of the value patient perspectives can bring to regulatory and HTA decision making.
- Identify the best approach to measure and report on the impact of patient-related evidence in regulatory and HTA decision making.
- Recommend areas for improvement to leverage patient-related evidence to support regulatory review and HTA decisions, including how best to measure.
- Provide input into the 2025 CIRS multi-stakeholder workshop entitled *Meaningful patient engagement in regulatory and HTA decision making – What are current practices and what impact does this have on the final assessment?*



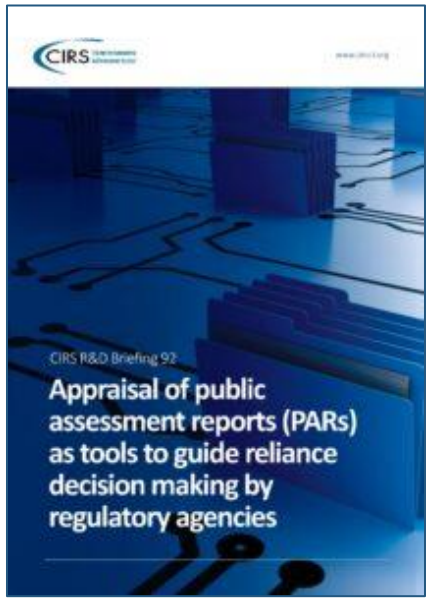
(Report available to CIRS member companies)

Publications

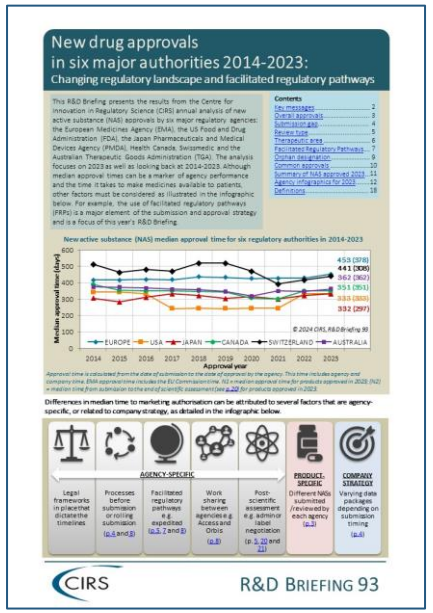
2024 R&D Briefings



CIRS R&D Briefing 91 – Approaches to implementing regulatory reliance: Considerations for agencies

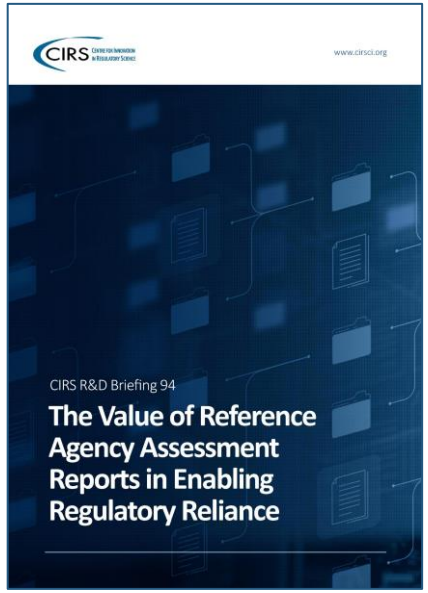


CIRS R&D Briefing 92 – Appraisal of public assessment reports (PARs) as tools to guide reliance decision making by regulatory agencies

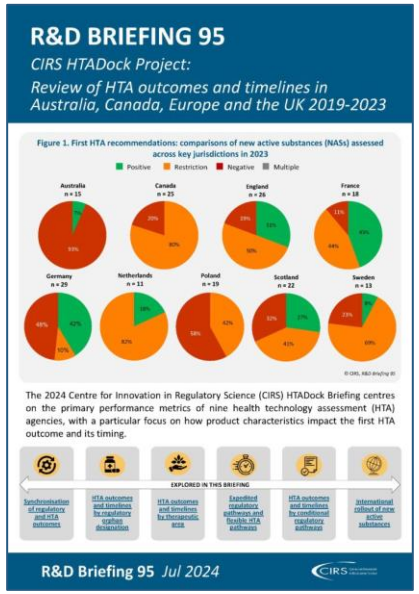


CIRS R&D Briefing 93 – New drug approvals by six major authorities 2014-2023: Changing regulatory landscape and facilitated regulatory pathways

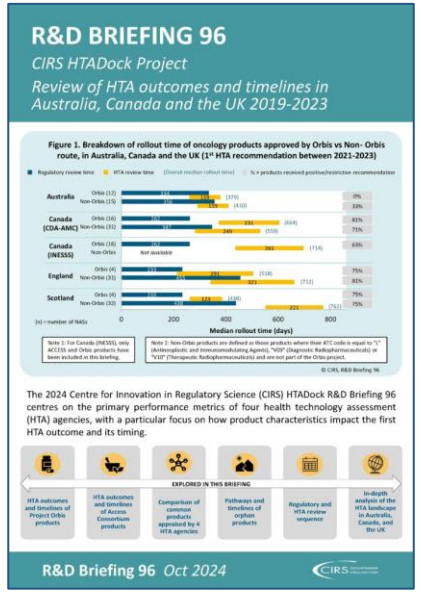
2024 R&D Briefings



CIRS R&D Briefing 94 – The value of reference agency assessment reports in enabling regulatory reliance

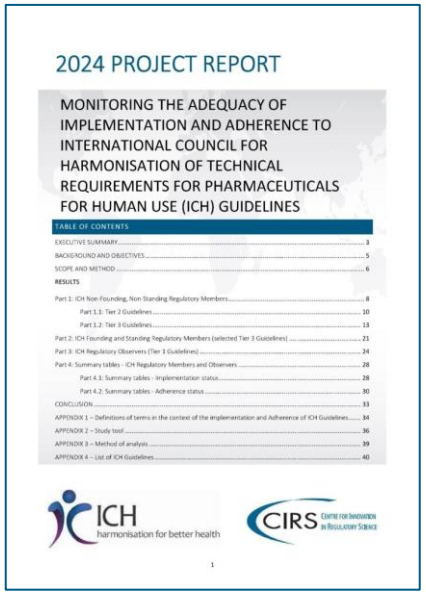


CIRS R&D Briefing 95 – CIRS HTADock Project: Review of HTA outcomes and timelines in Australia, Canada, Europe and the UK 2019-2023

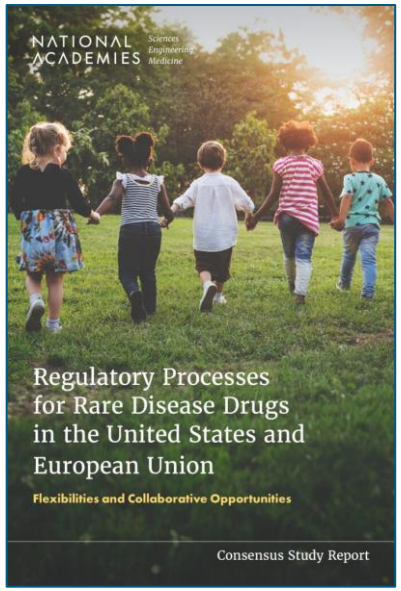


CIRS RD Briefing 96 – CIRS HTADock Project: Review of HTA outcomes and timelines in Australia, Canada and the UK 2019-2023

2024 Project reports with external partners



[Monitoring implementation and adherence to ICH guidelines]



[Regulatory processes for rare disease drugs in the US and EU]

[ICMRA Rare Disease Workshop Report]

2024 Journal Publications & Books

- Sithole T, Ngum N, Owusu-Asante M, **Walker S**, & Salek S (2024). Comparison of Three Regional Medicines Regulatory Harmonisation Initiatives in Africa: Opportunities for Improvement and Alignment. Int J Health Policy Manag. 2024;13:8070. doi:10.34172/ijhpm.2024.8070
- Ngum N, Ndomondo-Sigonda M, Habonimana R, Siyoi F, Irasabwa C, Ojukwu J, Apolinary F, Okello A, Ahmada S, **Walker S** and Salek S (2024) Evaluation of good review practices in member authorities of the East African Medicines Regulatory Harmonisation initiative: strategies for alignment with African medicines agency. Front Med (Lausanne). 2024;11:1437970. Published 2024 Aug 29. doi:10.3389/fmed.2024.1437970
- Ngum N, Ndomondo-Sigonda M, Habonimana R, Siyoi F, Irasabwa C, Ojukwu J, Apolinary F, Okello A, Ahmada S, **Walker S** and Salek S (2024) Evaluation of the Review Models and Approval Timelines of Agencies, participating in the East African Medicines Regulatory Harmonisation Initiative: Alignment and Strategies for Moving Forward. Front Med (Lausanne). 2024;11:1438041. Published 2024 Sep 17. doi:10.3389/fmed.2024.1438041
- Chisha CS, Siyanga M, Leigh S, Kermad A, **Walker S**. Evaluation of the Regulatory Review Process of the Zambia Medicines Regulatory Authority: Challenges and Opportunities. Ther Innov Regul Sci. Published online December 27, 2024. doi:10.1007/s43441-024-00730-6
- Ngum N, Salek S & **Walker, S**. (2024). The role of regional initiatives in the operationalisation of the African Medicines Agency: Contribution of the EAC-MRH initiative. [PhD thesis]

2024 Online articles/external blogs

- DIA Global Forum, September 2024 - Minding the Gap: CPP Utilisation Practices and the Impact on Submission Gap in Growth and Emerging Markets
- DIA Global Forum, November 2024 – Regulatory and Access Approaches for Vaccines: Recommendations from an Expert Workshop
- OHE Insights Blog - Is collaboration between and across regulatory and HTA agencies the answer to access challenges?

External presentations

2024 Conference presentations

21st February, EURORDIS Roundtable of Companies

- Dr Tina Wang – Speaker - Do development, review and reimbursement frameworks need adapting to improve evidence generation and financially sustainable access for rare disease products?

11-15th March, DIA Europe

- Dr Magda Bujar – Facilitator – Pre-Conference workshop on "Reliance for post-approval changes: How do we move from exceptional to routine use?"
- Dr Magda Bujar – Speaker – Regulatory risk-based approaches: Are these enabling better availability and access to medicines globally?

1st-5th April, BioHabana Congress – Regulatory Symposium

- Dr Mario Alanis– Speaker – Implementation of reliance

6-8th May, RAPS Euro Convergence

- Dr Magda Bujar - Chair – Emerging advancements in eCTD: A panel discussion on cloud integration, data standards and publishing perspectives in regulatory submissions

16-19th May, DIA China – ICH Day

- Dr Neil McAuslane – Speaker – Importance of building trust and capacity in international standards, guidelines and regulatory science

5-6th June, DIA Southeastern Europe Conference

- Dr Magda Bujar – Speaker – Feedback from studies: Regulatory risk-based approaches – Are these enabling better availability and access to medicines globally?

15-19th June, HTAi Annual Meeting

- Dr Belen Sola – Pre-recorded presentation – HTA timelines and outcomes of common compounds in France, Germany, Sweden and Poland from 2015-2023
- Dr Tina Wang - Poster – Navigating HTA Requirements During Development Through Early HTA Scientific Advice: Insights From Companies' Strategies, Challenges, And Priorities
- Dr Tina Wang – Poster - Rare Disease Product Approvals: The Changing Regulatory And HTA Landscape Between 2018-2022

16-20th June, DIA Global Annual Meeting

- Dr Magda Bujar – Chair – What is the value of reference agency assessment reports in enabling reliance and what do relying agencies require?
- Juan Lara - Poster – Assessing the Use of Risk-Based Approaches in Four Major Agencies – What is the Impact on the Approval of New Medicines?
- Lorraine Danks – Poster - The Economic Impact of Reliance on a National Regulatory Authority: a SAHPRA Case Study
- Mercy Owusu-Asante – Poster - Comparison of Regulatory Performance of WHO Maturity Level 3 National Medicines Regulatory Authorities (NMRAs) in Africa: Identifying Best Practices
- Nancy Ngum – Poster - Evaluation of Good Review Practices in Member Agencies of the East African Medicines Regulatory Harmonisation Initiative
- Constance Chisha – Poster - Assessment of the Regulatory Review Process of the Zambia Medicines Regulatory Authority: Opportunities and Challenges

25-26th September, DIA Latin America Annual Meeting

- Juan Lara - Speaker - Appraisal of Public Assessment Reports (PARs) as Tools to Guide Reliance Decision Making by Regulatory Agencies

17-20th November, ISPOR Europe

- Penelope Cervelo – Poster - Frequency and Variation of Clock-Stop During EMA Assessment for Oncology Products: Implication on JCA Timelines

25-27th November, DIA Middle East and North Africa

- Dr Neil McAuslane– Speaker - International Collaboration, Harmonisation & Convergence: ICH Implementation Survey Results

2024 Webinars

5th April, Pharma Group Vietnam
- Approaches to Implementing Regulatory Reliance: Considerations for Agencies

24th June, FPath Project
- What core information is needed for a reliance decision?

28th August, CIRS
- Utility of public and non-public assessment reports from reference agencies – What is being utilised and for what reason?

12th December, FPath Project
- How are Reference Agencies Evolving to Meet the Needs of the Evolving Regulatory Landscape?

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Appendix – About CIRS

CIRS is an experienced convening organisation with a global remit

Mission

To identify and apply scientific principles for the purpose of advancing regulatory and health technology assessment (HTA) policies and processes in developing and facilitating access to pharmaceutical products

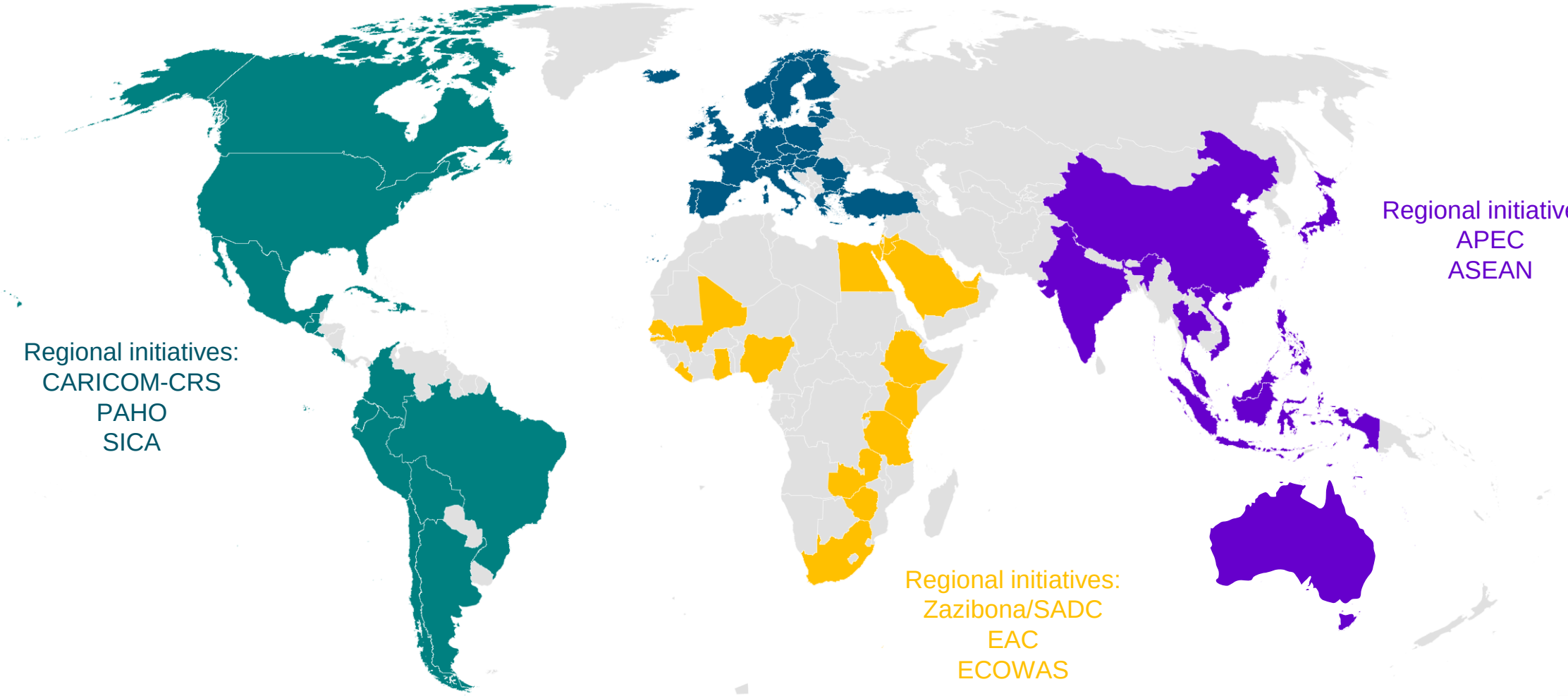
35+ yrs experience in bringing **global** industry, regulators, HTA bodies, payers, academics and others together in a **neutral** atmosphere to identify and address key issues in the development, licensing and reimbursement of medicines.

Subsidiary of Clarivate plc – **operate independently** as a non-profit. Financed by industry membership fees, special projects, grants e.g. from regulators, HTA bodies, Bill and Melinda Gates Foundation

See CIRS About Us

Companies of all sizes participate in CIRS

CIRS collaborates with regulators all over the world



CIRS works with HTA & coverage bodies in many jurisdictions

Non-Europe	Organisation
Argentina	Institute for Clinical Effectiveness and Health Policy
Australia	PBAC
Brazil	CONITEC
Canada	CADTH; INESSS, Alberta Health Services
China	National Centre for Medicine and Technology Assessment
Chinese Taipei	Division of HTA, CDE
Colombia	IETS
Malaysia	MoH
Singapore	ACE
Thailand	HITAP
USA	UnitedHealth Group; Blue Cross/Blue Shield Association; Kaiser Permanente Institute for Health Policy; AHRQ; OPTUM

Europe	Organisation
Austria	Association of Austrian Social Insurance Institutions
Belgium	INAMI; KCE
Bulgaria	NCPR
Croatia	AAZ
Denmark	DKMA
England, Wales	NICE
Finland	THL
France	HAS
Germany	G-BA, DAK-Gesundheit
Ireland	NCPE
Netherlands	ZIN
Norway	NoMA
Poland	AOTMiT
Portugal	INFARMED
Romania	NAMMDR
Scotland	SMC
Spain	AEMPS, MoH
Sweden	TLV
Switzerland	BAG

Three pillars of CIRS activities – Foundation of CIRS research themes

Thank you!

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