Aligning global value-based decision making

THE CIRS 2021 AGENDA

CONSENSUS • TRUST • ACCESS

CIRS Centre for Innovation in Regulatory Science

About CIRS

Mission

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 in developing and facilitating access to pharmaceutical products

How we operate

The Centre for Innovation in Regulatory Science (CIRS) is a neutral, independently managed UK-based subsidiary of Clarivate plc. We operate as a not-for-profit organisation, deriving funding from membership dues, special projects and grants to cover our operating and research costs.

We are governed by our own dedicated advisory boards made up of external international experts from academia, industry, regulatory agencies and HTA bodies. Our Scientific Advisory Council (SAC) and HTA Steering Committee advise on workshop topics and content, as well as our research programme.

What makes us unique

What sets us apart is our ability to bring **global** industry, regulators, HTA bodies, payers and academics together in a **neutral** atmosphere to identify and address key issues in the development, licensing and reimbursement of new medicines. We have been doing this for over 35 years through **focused** meetings and collaborative research.

Our workshops have consistently received positive feedback ratings of over 90% and resulted in recommendations that inform strategic and policy level thinking. The strong support for our research as well as attendance in meetings demonstrates the trust and confidence our stakeholders have in us.

We are also **evidence-driven** and **transparent** in our work. The data we collect are used to support our workshops and we endeavour to make these publicly available through peer-reviewed journals or our R&D Briefings.

Our small but dedicated team of experienced scientists strive to ensure that the needs and priorities of our stakeholders are at the heart of CIRS activities. Our door is always open to new ideas and suggestions that fit with our mission.

CIRS team, January 2020

Three pillars of CIRS activities

Metrics | Quality | Alignment

Metrics - evidence-driven insights into company and agency performance

CIRS provides unique insights and an independent viewpoint on the performance of companies, regulatory and HTA agencies through its metrics programmes. Data are collected and analysed to give a better understanding of regulatory and HTA assessment times and evidentiary requirements. This form of benchmarking details regulatory and HTA processes and practices, identifies where improvements can be made and informs company and agency strategies.

Quality– improving decision-making processes during the medical product life cycle

CIRS works with companies and agencies to evaluate the quality of their processes, identify challenges, and implement best practices. Tools developed from these projects support decision-making practices and their documentation in general or are specific to areas such as benefit-risk assessment. The aim is to foster learning and improve transparency, predictability and consistency of critical decisions during development, regulatory review and health technology assessment.

Alignment– converging stakeholder priorities and processes to accelerate patient access

Through collaborative research projects and multi-stakeholder workshops, CIRS promotes harmonisation and alignment across HTA and regulatory agencies, as well as between HTAs and regulators themselves. Not only does this facilitate capacity building but also helps to enable more efficient and effective development of medicines.

Attendees at the 2019 Annual OpERA Regulatory Forum in Singapore

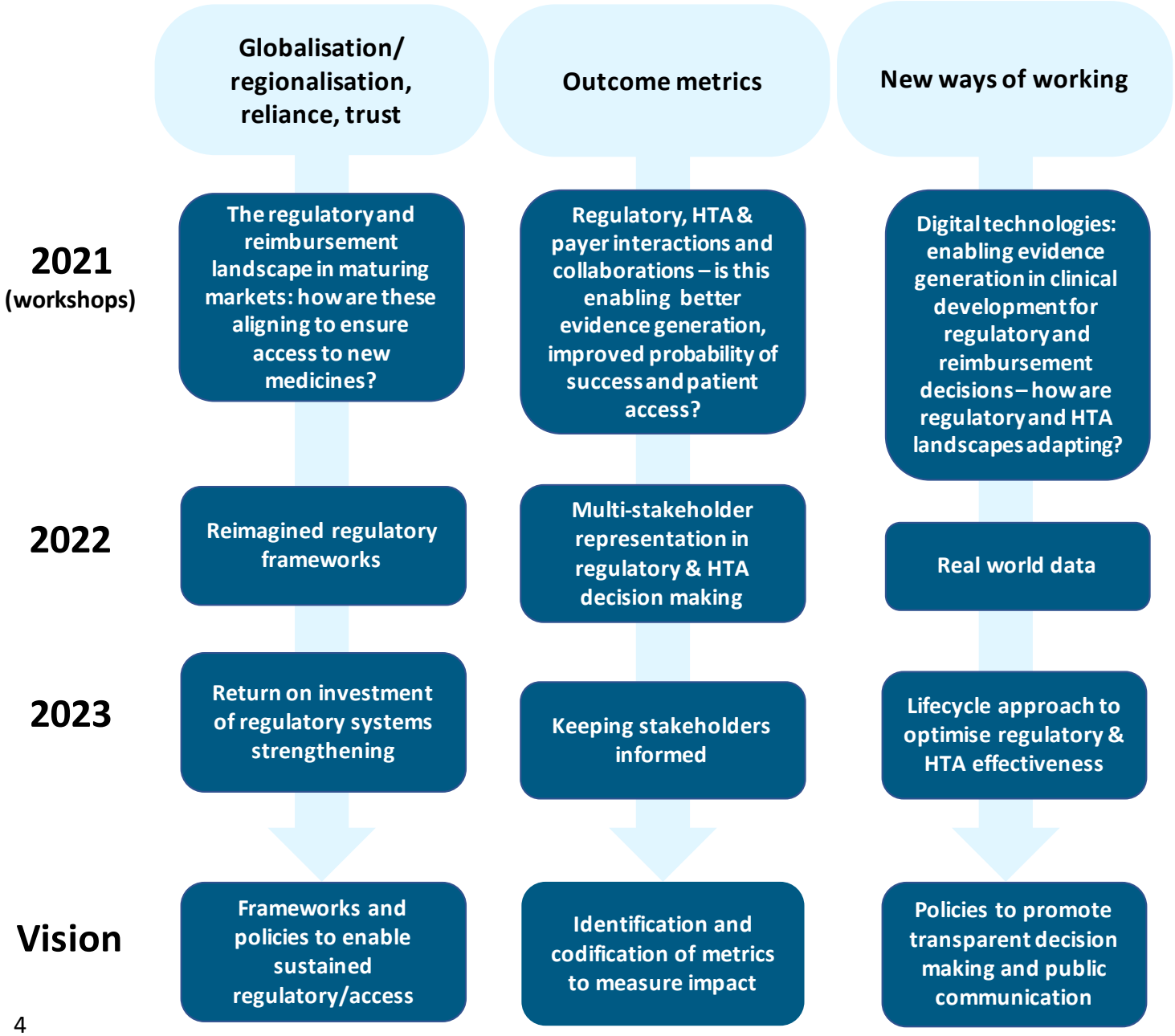
Research strategy 2021-2023

CIRS sets its three-year research strategy with formal input from the Scientific Advisory Council (SAC) and HTA Steering Committee, as well as ad hoc feedback from companies and agencies. Our 2021-2023 programme, which will be achieved through workshops, fora and research projects, is grouped into three strategic themes:

Globalisation/regionalisation, reliance, trust - This theme is focused upon how organisations are evolving in order to ensure sustainability in terms of approval/access to new medicines. As well as ensuring a performance management culture, this theme incorporates a number of inter-related aspects such as regulatory strengthening, re-imagined regulatory frameworks and the alignment of regulatory and HTA.

Outcome metrics - CIRS has the experience of benchmarking metrics going back decades and this theme aims to continue and extend that solid foundation. This will contain outcome measures as well as the identification and codification of metrics to measure impact. A key focus for 2021-2023 will be developing and utilising metrics on efficiency and effectiveness of process and patient involvement in regulatory and reimbursement decision making.

New ways of working - The COVID-19 pandemic has not only challenged regulatory and HTA systems globally to work in new ways but has also accelerated changes in the digital space. We will examine these new ways of working across industry, regulatory and HTA with an emphasis on digital – notably evidence generation, the Cloud and digital therapeutics – and new product focus including ATMPs. This will be aimed at the intersection of CIRS' core areas which include policy and the alignment of regulatory and HTA, as well as global versus regional aspects.



Member companies and participating authorities

Member Companies

USA	Europe	Japan
AbbVie	AstraZeneca	Astellas
Amgen	Bayer	Eisai
Biogen	GlaxoSmithKline	Takeda
CSL Behring	Ipsen	
Eli Lilly and Co.	Leo	
Johnson & Johnson	Lundbeck	
Merck & Co	Novartis	
Pfizer	Roche	
	Sanofi	
	UCB	

HTA and Coverage Bodies

Country	Organisation
Australia	PBAC
Belgium	INAMI; KCE
Brazil	CONITEC
Canada	CADTH; DSEN, Canadian Institutes of Health Research, INESSS, Alberta Health Services
Croatia	AAZ
Denmark	DKMA
England, Wales	NICE
Europe	EUnetHTA
Finland	THL
France	HAS
Germany	G-BA, AOK-Bundesverband
Lithuania	VASPVT
Norway	NOKC
Poland	AHTAPol
Portugal	INFARMED
Scotland	Scottish Medicines Consortium
Singapore	ACE
Spain	CAHIAQ, Osteba
Sweden	TLV
Switzerland	BAG
The Netherlands	ZIN
United States	United Health Group; TEC, Blue Cross/Blue Shield Association; Kaiser Permanente Institute for Health Policy; AHRQ; OPTUM

Participating Regulatory Authorities

Asia - Country	Authority
Australia	TGA
China	NMPA
Chinese Taipei	TFDA; CDE
India	CDSCO
Indonesia	NADFC
Japan	MHLW, PMDA
Malaysia	NPRA
Philippines	PFDA
Singapore	HSA
South Korea	MFDS
Thailand	Thai FDA
Vietnam	DAV
Regional initiatives	APEC

Participating Regulatory Authorities

Americas - Country	Authority
Argentina	ANMAT
Brazil	ANVISA
Canada	Health Canada
Chile	ANAMED
Colombia	INVIMA
Cuba	CECMED
Haiti	DPM/MT-MSPP
Mexico	COFEPRIS
Peru	DIGEMID
United States	FDA
Regional initiatives	CARICOM-CRS/PAHO

EME - Country	Authority
Denmark	DKMA
EU	EMA
Ireland	HPRA
Israel	MoH
Jordan	JFDA
Kuwait	KDFC
Oman	MoH
Qatar	SCH
Saudi Arabia	SFDA
Sweden	MPA
Switzerland	Swissmedic
The Netherlands	MEB
Turkey	TITCK
United Arab Emirates	MoH
United Kingdom	MHRA
Regional initiatives	GHC

Africa - Country	Authority
Botswana	BoMRA
Burkina Faso	DGPML
Ethiopia	EFDA
Gambia	MCA
Ghana	FDAG
Kenya	PPB
Mozambique	MoH
Namibia	NMRC
Nigeria	NAFDAC
Senegal	MoHP
South Africa	SAHPRA
Tanzania	TMMDA
Zambia	ZAMRA
Zimbabwe	MCAZ
Regional initiatives	AMRH-EAC, Zazibona/ SADC, WAHO

CIRS Committees

CIRS is governed by its own dedicated advisory boards made up of external international experts from academia, industry, regulatory agencies and HTA bodies. The Scientific Advisory Council (SAC) and HTA Steering Committee advise CIRS on workshops, special projects, publications as well as the research programme, to ensure neutrality and that the areas are unbiased and the programme meets the needs to all stakeholders. The selection of Committee Members and Chairs is set out in the Terms of Reference.

Scientific Advisory Council

Chair: Adjunct Prof John Skerritt, Deputy Secretary for Health Products Regulation, Department of Health, Canberra, Australia **Vice-Chair: Prof Hans-Georg Eichler,** Senior Medical Officer, EMA

Dr Claus Bolte, Head of Sector Marketing Authorisation, Swissmedic
Dr Harald Enzmann, Chair, CHMP/ EMA
Dr Ian Hudson, Senior Advisor, Integrated Development, Global Health, Bill and Melinda Gates Foundation, UK
Prof John Lim, Executive Director of CoRE, Duke-NUS Medical School and Chairman, Consortium for Clinical Research & Innovation Singapore
Dr Theresa Mullin, Director, Office of Strategic Programs, US FDA, CDER
Dr Brian O'Rourke, Former CEO and President, CADTH, Canada
Dr Junko Sato, Office Director, Office of International Program, Pharmaceuticals and Medical Devices Agency (PMDA), Japan
Dr Xie Songmei, Deputy Director of Clinical Department Center for Drug Evaluation, NMPA, China
Dr John Patrick Stewart, Director General, Therapeutic Products Directorate, Health Canada

Deborah Autor, Global Head of Regulatory Excellence, AstraZeneca, USA
Dr Fabio Bisordi, Global Head International Regulatory Policy, F.Hoffmann-La Roche Ltd
Dr Tim Garnett, CMO, SVP, Eli Lilly
Adrian Griffin, Vice President for HTA Policy, Johnson & Johnson
Dr Peter Honig, Senior Vice President and Head of Worldwide Safety and Regulatory, Pfizer
Dr David Jefferys, SVP, Head of Global Regulatory, Eisai Europe Ltd
Dr Sabine Luik, Chief Medical Officer and SVP, Global Medical, Regulatory & Quality, GlaxoSmithKline
Dr Roopal Thakkar, Vice President, Regulatory Affairs and R&D Quality Assurance, Abbvie
Dr Max Wegner, Head Regulatory Affairs PH & CH, Bayer AG
Dr Neil McAuslane, Director, CIRS
Dr Lawrence Liberti, Head, Regulatory Collaborations, CIRS
Prof Stuart Walker, Founder, CIRS

Specialist Advisors to the Executive Director

Dr Thomas Lönngren, Former Executive Director, EMA
Dr Murray Lumpkin, Deputy Director, Integrated Development, and Lead for Global Regulatory Systems Initiatives, Bill and Melinda Gates Foundation
Prof Mamoru Narukawa, Associate Professor Pharmaceutical Medicine, Kitasato University Graduate School of Pharmaceutical Sciences, Tokyo, Japan
Dr Tomas Salmonson, Former Chair, CHMP/EMA
Dr Joseph Scheeren, President, CEO, Critical Path Institute

HTA Steering committee

Chair: Dr Brian O'Rourke, Former CEO and President, CADTH, Canada

Vice-Chairman: Prof Adrian Towse, Director Emeritus and Senior Research Fellow, OHE

Dr Nick Crabb, Programme Director, Scientific Affairs, NICE

Prof Hans-Georg Eichler, Senior Medical Officer, EMA

Dr Wim Goettsch, Associate Professor HTA, Utrecht University; Special Advisor HTA, ZIN

Evert Jan van Lente, Director EU-Affairs, AOK-Bundesverband

Niklas Hedberg, Chief Pharmacist, TLV

Andrew Mitchell, Strategic Adviser, DoHA

Adrian Griffin, Vice President for HTA Policy, Johnson & Johnson

Dr Michael Happich, Director, BioMed HTA, Eli Lilly and Co

Dr Adam Heathfield, Senior Director, Patient and Health Impact Innovation Centre, Pfizer

Dr Maria Kubin, Head, Integrated Evidence Planning for the Cardiovascular Therapy Area, Bayer

Dr Vanessa Elisabeth Schaub, Global Access Senior Health Systems Strategy Leader HTA & Reimbursement, Roche

Dr Sean Tunis, Principal, Rubix Health and Senior Advisor, FDA

Prof Finn Børlum Kristensen, Former EUnetHTA Executive Committee Chairman and EUnetHTA Secretariat Director, Faculty of Health Sciences, University of Southern Denmark

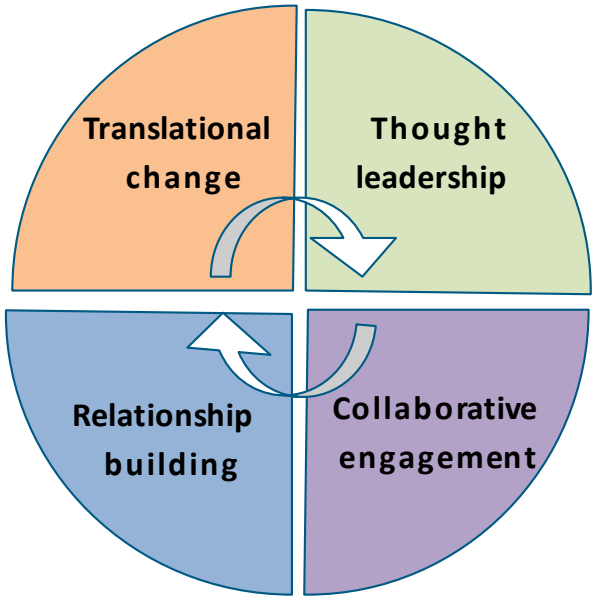
Dr Lawrence Liberti, Head, Regulatory Collaborations, CIRS

Dr Neil McAuslane, Director, CIRS

Tina Wang, Manager, HTA Programme, CIRS

CIRS Workshops

Our workshops are small, international meetings that provide exceptional learning and networking opportunities. Participants can interact with peers from industry, agencies and academia in a neutral atmosphere of informed and productive discussion to produce recommendations to move important topics forward in the development, regulation and reimbursement of medicines.



Workshop topics are aligned with our research priorities (see page 4) and frequently build on the recommendations of previous meetings to continue to develop strategic and policy-level thinking. We usually hold three workshops a year, with each one addressing one of the following areas:

1. Global development e.g. reliance, effectiveness, efficiency
2. Current 'hot' topic e.g. digital, real-world data, decision making, new ways of working
3. Regulatory-HTA alignment e.g. early scientific advice

WHAT OUR STAKEHOLDERS SAY

"CIRS workshops provide an excellent opportunity to meet top management from EMA and FDA. Good open discussion between stakeholders that I have not experienced before."
Pharmaceutical company

"The overall quality of discussions at CIRS workshops is excellent, the engagement with and by the HTA/payer community is impressive and sets a nice course forward"
HTA agency

Our workshops receive consistently high feedback scores

All our workshops feature interactive syndicate sessions that result in a set of recommendations. Here's an example from one of the syndicates at CIRS Workshop, *Identifying and understanding regulatory and reimbursement uncertainty in development: how can this improve predictability of regulatory and HTA outcomes?* 9-10 October 2019, Surrey, UK.

Recommendations on an Integrated Uncertainty Management Plan (IUMP) framework:

- An IUMP should be a 'living' framework that can be used over the medical product life cycle and should be produced by the company and discussed with stakeholders in the context of regulatory and HTA advice.
- To inform the development of an IUMP, there needs to be a mapping exercise on current context, such as mapping existing stakeholder models, payer/HTA systems and advice pathways.
- In the longer term, there need to be discussions to refine the IUMP scope and methodology.

2021 Workshops

10-11 March 2021, Runnymede Hotel, Surrey, UK

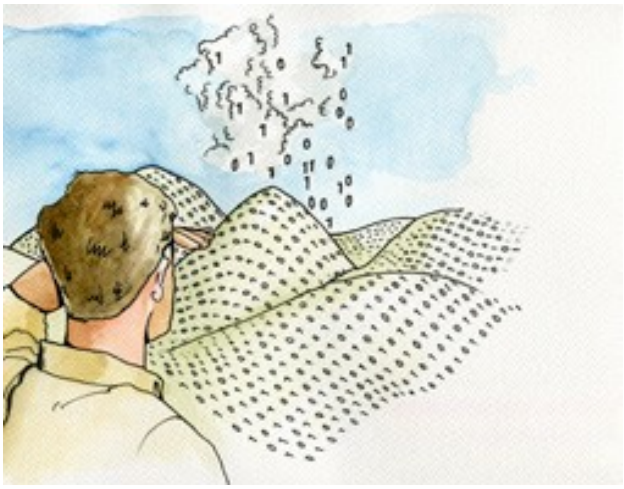
Regulatory, HTA and payer interactions and collaborations – Is this enabling better evidence generation, improved probability of success and patient access?

OBJECTIVES

- Discuss the current and future of regulatory, HTA and payer initiatives within and across jurisdictions
- Identify through case studies the key areas and types of interactions and collaborations that are seen as effective models
- Understand the benefit these interactions between stakeholders bring to enabling improved decision making by different stakeholders and what can be learnt across jurisdictions

Key discussion points:

- How the level and depth of interactions between stakeholders in development is changing and the future of direction of travel for such interactions?
- What are the key areas where there are divergences in evidence needs and which interactions are of value to improve decision making?



24-25 June, Tyson's Corner, Virginia, USA

Digital technologies: enabling evidence generation in clinical development for regulatory and reimbursement decisions - how are the regulatory and HTA landscapes adapting?

OBJECTIVES

- Discuss how agencies and companies are currently developing the role of digital technology for evidence generation in clinical development for regulatory and HTA decision making
- Identify the opportunities and how to reduce potential barriers going forward for evidence generated by digital technologies for use in the review and reimbursement of medicines
- Recommend areas of work/research which could enable ways that will allow alignment across jurisdictions to ensure digital technologies maximise their potential within a fit for purpose regulatory and HTA environment

Key discussion points:

- How digitisation and digital health technologies are transforming healthcare and how companies, regulatory and HTA agencies are looking to derive actionable insights from its utilisation?
- What are the opportunities to shape the environment both pre- and post-approval so as to reduce potential barriers going forward

September/October, Location TBC

The regulatory and reimbursement landscape in maturing markets: how are these aligning to ensure both availability and access to new medicines?

OBJECTIVES

- Discuss the current and future regulatory and HTA landscape within maturing jurisdictions and how or if these are aligned.
- Identify through case studies different models and maturity of systems within countries as well as the challenges and opportunities.
- Make recommendations on what can be learnt across jurisdictions from the current initiatives so as to inform the future evolution of the regulatory-HTA landscape in maturing countries.

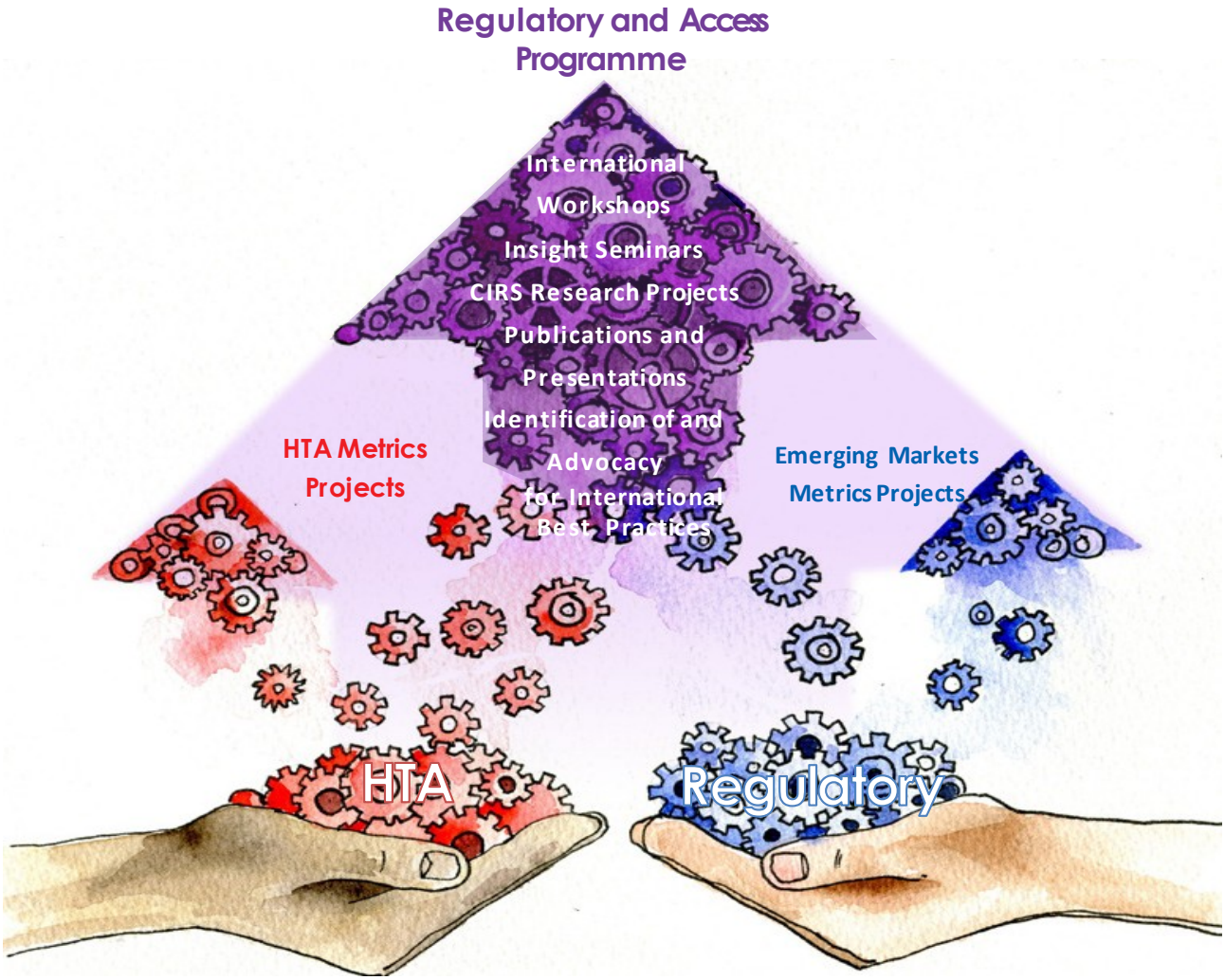
Key discussion points:

- How are HTA processes and practices evolving in countries with maturing markets
- What are the areas of alignment between the regulatory process and HTA mechanisms to ensure not just timely availability of new medicines but also access

Membership to the **CIRS Regulatory and Access programme** is open to all pharmaceutical companies, particularly those engaged in research and development of new active substances, prescription medicines, devices and biologics with a view to global product development, regulatory affairs and market access.

By becoming a member, your company can support CIRS' mission, participate in multi-stakeholder activities such as workshops, research and benchmarking metrics projects, benefit from priority access to CIRS publications and become part of an international community to help shape major policy topics. These benefits are described in more detail on the following page.

If your company would like to find out more about becoming a CIRS member, please contact Gill Hepton: ghepton@cirsci.org



Why become a CIRS member?

Be part of a global network

The CIRS community involves regulators, HTA agencies, payers, industry and academia from around the world.

By becoming a CIRS member, you can interact with these stakeholders at small, productive meetings* including:

- Multi-stakeholder workshops (see p7)
- Annual industry-focused technical forums
- Ad hoc industry-focused webinars**

Access to insights & knowledge

As well as gaining exclusive access to the results of research that your company has contributed to, as a CIRS member you will also be able to access:

- CIRS Members website - designed to be a 'one-stop shop' for CIRS resources including workshop slides, R&D Briefings and open access publications
- CIRS Regulatory & Reimbursement Atlas™ - an online tool that maps regulatory, HTA and payer pathways for more than 70 jurisdictions around the world.

In addition, CIRS members have early access to R&D Briefings including two annual Briefings focusing on regulatory and HTA agency benchmarking of new active substances. Additional benefits include:

- Exclusive access to the slides from the Briefing
- Exclusive analysis of your company's performance compared to overall benchmarks**
- Industry-wide webinar to review key findings

Participate in research & metrics

CIRS membership offers several opportunities to participate in research that gives unique insights into the regulatory and access landscape, including:

- Annual focus studies
- Emerging Markets Metrics Programme (for an additional fee) – through this annual study CIRS analyses company-provided data on time to submissions and approvals for 18 Emerging Market regulatory authorities. More information can be found on p11.
- HTA Metrics Programme (for an additional fee) - through this annual study CIRS tracks company-provided data to quantify the impact of addressing HTA requirements on clinical development programmes, reimbursement timing and outcomes in Europe, Australia and Canada. More information can be found on p11.
- Special Projects – CIRS has worked with companies on ad hoc projects that answer short business questions, produce internal strategy documents or facilitate external advocacy. To find out more, please contact mbujar@cirsci.org

Contribute to research & advocacy

CIRS membership helps to support the CIRS research programme, including PhD projects and the development of tools such as the CIRS Regulatory and Reimbursement Atlas™ and other tools that promote quality of decision making and regulatory and HTA alignment. In addition, membership supports CIRS' advocacy activities including the organisation of meetings with regulatory and HTA agencies.

By being a member, you can contribute to the direction of CIRS advocacy and research and put forward subjects for discussion at workshops, as well as topics for surveys and studies. Individuals from member companies can also be nominated to join CIRS committees (see p6).

*Full registration and accommodation (excluding travel) for two participants at each workshop and registration for one person to each of the annual forums (accommodation not included).

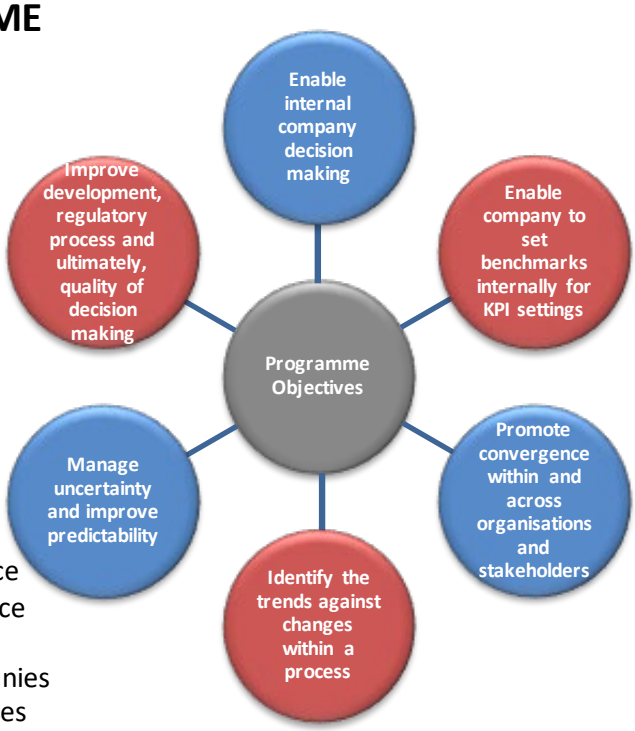
**New for 2021 membership

Join our industry metrics programmes

As well as the **Regulatory and Access programme,** CIRS offers two industry metrics programmes that are available as add-on benefits to CIRS members.

EMERGING MARKETS (EM) METRICS PROGRAMME

Globalisation of the world's pharmaceutical markets means that quality information for development and registration of new medicines in EM countries is more important than ever before. The CIRS EM Metrics Programme can help you to get ahead in these fast-growing markets by providing comparative data and information on the evolving regulatory environment at the country and regional level.



The Programme collects company data on submission, approval and rollout times in 18 countries and one regional alignment initiative across Asia, Latin America, Europe, Middle East and Africa. The data is analysed, aggregated and anonymised, resulting in an industry-wide picture of the regulatory landscape in each country, which you can then compare your company against.

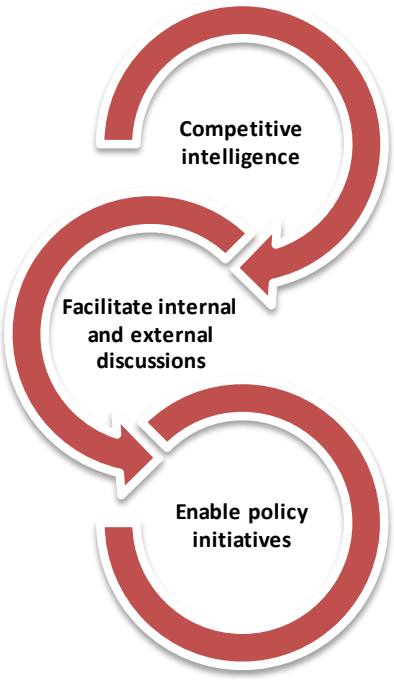
Annual deliverables of the Programme include:

- Main report, aimed at Global Regulatory Leads, Policy and Intelligence
- Executive summary, aimed at Management and Policy and Intelligence
- Country-specific summaries, aimed at Regional Affiliates
- Results of a focused study on a topic of interest to participant companies
- Industry Discussion Meeting to review trends and discuss new analyses
- Periodic updates on the Programme and CIRS advocacy activities

If your company would like to find out more about joining the EM Metrics Programme, please contact Prisha Patel: ppatel@cirsci.org

HEALTH TECHNOLOGY ASSESSMENT (HTA) METRICS PROGRAMME

HTA is a major challenge in drug development and market access, as requirements vary by jurisdiction and there is an overall lack of predictability and transparency in the HTA process. The CIRS HTA Metrics Programme can help your company to mitigate these risks and plan for success by providing unique insights and actionable benchmarks.



The Programme collects company data on individual products from development through to rollout in Australia, Canada, England, France, Germany, Italy and Spain. The data is analysed, aggregated and anonymised, resulting in an industry-wide picture of the HTA landscape that you can compare your company against. Key analyses address important business questions such as types and outcomes of early HTA-related advice and inclusion of active comparators during development and the acceptance by HTA agencies.

Annual deliverables of the Programme include:

- Executive summary and company-specific report
- Country-specific summaries
- Results of a focused study on a topic of interest to participant companies
- Industry Discussion Meeting to review trends and discuss potential for new analyses
- Periodic updates on the Programme and CIRS advocacy activities

If your company would like to find out more about joining the HTA Metrics Programme, please contact Tina Wang: twang@cirsci.org

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 HTA policies and processes. CIRS provides an international forum for industry, regulators, HTA and other healthcare stakeholders to meet, debate and develop regulatory and reimbursement policy through the innovative application of regulatory science and to facilitate access to pharmaceutical products. 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, special projects and grants.

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CIRS Centre for Innovation in Regulatory Science