WEBINAR REPORT

Transforming Real-World Evidence with AI: From Data to Decisions

Wednesday, 15 October 2025, 15:00-16:30 CEST

Speakers

JULIÁN ISLA
Founder, Foundation 29
Data and AI Resource Manager, Microsoft
EURORDIS Member

JING WANG-SILVANTO
Senior Director Global Value Evidence, Astellas

STEPHEN DUFFIELD
Associate Director Real-World Evidence Methods, NICE

FARAH HUSEIN
Director Science and Methods, Canada's Drug Agency (CDA-AMC)

Co-Moderators

NIKLAS HEDBERG
Chief Pharmacist, TLV; Co-Chair of the HTA Coordination Group

KAREN FACEY
RWE4Decisions Facilitator

TABLE OF CONTENTS

INTRODUCTION

The 15 October RWE4Decisions Webinar *"**Transforming Real-World Evidence with AI: From Data to Decisions**"* gathered a diverse and engaged audience of 200+ participants to explore how artificial intelligence (AI) can enhance the generation of real-world evidence (RWE) to support better decision-making across the healthcare ecosystem. Co-moderated by Niklas Hedberg (TLV) and Karen Facey (RWE4Decisions), the session brought together perspectives from health technology assessment (HTA) bodies, AI specialists, patient representatives, and industry. Speakers reflected on the opportunities and challenges of integrating AI into evidence generation, emphasising the importance of human oversight, trust, and collaboration. The discussion highlighted practical uses of AI from different stakeholders' perspectives, showing various adoption levels and challenges across organisations, and the need for shared frameworks to ensure robust, transparent, and ethically grounded AI-enabled RWE.

HTA BODIES NAVIGATING AI: REFLECTIONS FROM TLV

Niklas Hedberg, Chief Pharmacist at the Dental and Pharmaceutical Benefits Agency in Sweden (TLV), Co-Chair of the Member State Coordination Group on HTA, and co-moderator of the webinar, opened the session with a reflection on the current state of AI adoption within HTA bodies. He emphasised the importance of listening to diverse stakeholder perspectives to understand the evolving role of AI in the healthcare ecosystem.

There is variability in national regulations, and this has implications for public bodies. So, although AI is not yet being used to make HTA decisions directly, it is increasingly being explored to expedite HTA processes. Examples of use in HTA bodies include for education, guidelines, and pilot projects, but each are progressing at different speeds depending on institutional priorities.

Niklas' reflections set the tone for the webinar, framing AI not as a distant future technology, but as a present-day tool with transformative potential, provided it is approached with ethical consideration and cross-sector collaboration.

AI'S TRANSFORMATIVE POTENTIAL FOR PATIENTS

Julián Isla (Data and AI Resource Manager at Microsoft and Founder of the Foundation 29) brought a unique dual perspective to the discussion, drawing on his professional expertise in software engineering and his personal experience as a rare disease patient advocate. His reflections underscored the disconnection between healthcare and other industries when it comes to using data to create continuous feedback that supports quality improvement. Unlike sectors such as aviation, healthcare remains fragmented, with limited mechanisms to track outcomes and close the loop between treatment and impact.

Julián illustrated this gap with a personal example: during an HTA evaluation for a treatment relevant to his son's condition, he asked the Spanish Ministry of Health for the actual cost of the disease to the system. The response revealed a reliance on artificial economic models that failed to account for real-life burdens, such as lost income, missed career opportunities, and unpaid caregiving. This case exemplifies the limitations of current evidence-generation practices.

While clinical trials remain the gold standard, they often fall short in capturing the broader consequences of treatment effectiveness. AI, particularly large language models, offers a promising solution by enabling the processing of vast and complex datasets and transforming real-world data into actionable real-world evidence.

THE INDUSTRY'S USE OF AI FOR REAL-WORLD EVIDENCE GENERATION

Jing Wang-Silvanto, Senior Director Global Value Evidence at Astellas, delivered a first keynote address that examined the strategic role of AI in transforming RWE generation across the medicine's life cycle and the broader healthcare ecosystem. She framed AI as a powerful tool to address long-standing challenges in data connectivity, regulatory compliance, and evidence generation, particularly in the context of accelerating access to innovative treatments.

She began by outlining the external and internal pressures facing the pharmaceutical sector, including increasingly complex regulatory demands, short timelines for evidence review, and intensifying competition. Data are often abundant but fragmented and poorly integrated, hence not usable for decision-making. However, AI offers a pathway to overcome these barriers by automating data processing, enabling predictive modelling, and uncovering insights from previously untapped sources, especially for rare diseases.

An article published in Nature on "*The next generation of evidence-based medicines*"[1] used the metaphor of an iceberg to illustrate the current state of evidence use: while clinical trial data mostly forms the visible tip, a vast reservoir of real-world and unstructured data lies beneath the surface, largely unexploited. AI could help unlock this hidden potential and support more holistic decision-making.

However, the promise of AI is not yet matched by its impact. A recent MIT study, highlighted that despite billions in investment, 95% of organisations report no return on AI adoption. This may be due to a lack of strategic implementation and assessment of impacts, Dr. Wang-Silvanto proposed a **four-level AI adoption maturity model** to assess organisational readiness to get tangible returns from AI, from exploratory pilots (level 1) to scaled enterprise-wide integration (level 4).

[1] Subbiah, V. The next generation of evidence-based medicine. Nature Medicine 29, 49–58 (2023). https://doi.org/10.1038/s41591-022-02160-z

Measuring returns by adoption levels – From Emerging (piloting) to Transformation (scaled)

Category	Level 1 – Emerging	Level 2 – Moderate	Level 3 – Advanced	Level 4 – Leading/Scaled
Workflow embedment	• Scattered pilots • Mostly exploratory	• Data foundation programs underway	• AI embedded across RWE functions	• AI at scale across RWE pipeline
Governance, validation & acceptance	• No enterprise-wide data governance • No standardized validation	• Internal governance body established	• Transparent validation frameworks	• Regulators accept AI-derived evidence
Workforce & skillset	• Skills limited to a few specialists	• Initial workforce training rolled out	• Multi-functional AI centers of excellence	• Continuous learning system with live RWE feeds
Decision making & impact	• No/limited impact • External AI experts engaged	• Pilot regulatory/scientific engagement	• Active collaborations with regulators & consortia	• Demonstrated impact on development & outcomes

Most organisations remain at level 1 or 2, with limited governance, validation, and internal expertise. Technology itself is not the barrier. Rather, success depends on the adoption levels within an organisation, the quality of data used in AI, the transparency of AI use, and the trust and acceptance of the AI outputs.

Potential **solutions** to these challenges, calling for open dialogue and collaboration among stakeholders (including patients and industry) and regulatory bodies to build transparency and trust. Furthermore, there is a need for guidance on how to validate AI outputs and the importance to establish robust data foundations.

HTA BODIES NAVIGATING AI: NICE'S POSITION STATEMENT

Stephen Duffield, Associate Director Real-World Evidence Methods at the National Institute for Health Care Excellence (NICE), delivered a keynote address outlining how the UK HTA body is approaching the integration of AI into HTA processes. He emphasised that AI is already influencing every stage of evidence generation, from data collection to evidence synthesis, and that NICE is actively adapting to this shift.
Acknowledging the **maturity scale** presented by Jing Wang-Silvanto, Stephen noted that NICE currently places itself between level 1 and 2, indicating early-stage implementation with some structured use cases but limited integration.

NICE's position statement on using AI in evidence generation outlines the HTA body's commitment to developing best practices standards and sets out three **priority areas**:

- Use of AI in evidence submitted to HTA bodies
- Evaluation of AI technologies
- Internal uses of AI to improve NICE's own processes

Transparency is key and NICE now requires submitters to declare and justify any use of AI in their evidence submission. The organisation encourages validation studies to accompany such submissions, particularly when AI plays a pivotal role. At this stage, AI should be used to augment human decision-making, not replace it.

Stephen concluded by stressing the need for ongoing piloting and collaboration, particularly through NICE's **HTA Lab**, which provides a regulatory sandbox for testing new methods.

HTA BODIES NAVIGATING AI: REFLECTION FROM THE CANADA'S DRUG AGENCY

Farah Husein, Director Science at Methods at the Canada's Drug Agency (CDA-AMC, formerly known as CADTH) offered her reflections on the use of AI for HTA.

This is a rapidly evolving area, and a shared steep learning curve across jurisdictions, but if NICE considers itself at level 1 or 2 of AI adoption, many others may be even earlier in their journey.

CDA-AMC has recently adopted the principles of NICE's position statement on AI and adapted it to emphasise the importance of **ethical principles** in guiding AI use: promoting human well-being, fostering accountability, ensuring inclusiveness and equity, and supporting responsiveness and sustainability. AI-generated evidence reflects the **biases** of its training data and algorithms, which raises concerns about representation, governance, privacy, and data sovereignty, especially in open-loop systems. These ethical considerations are not just theoretical; they apply equally to CDA-AMC's own internal use of AI for businesses efficiencies or process improvements.

Transparency and **credibility** are key. It is essential to clearly document what was done when AI was used in evidence generation, how it was done, and why it was done. The "human in the loop" - human involvement is critical to ensure that what is produced is the same quality as what our most experienced individuals would produce.

Finally, Farah highlighted the importance of **alignment across jurisdictions** and other interested and impacted groups given that evidence is rarely generated for a single market, echoing Niklas' reflections.

PANEL DISCUSSION

A **panel discussion** followed, where the speakers responded to the following questions from the audience and co-moderators:

- If the returns for AI were real, we would already be seeing mass layoffs in these functions. Why are we not seeing this yet? Or are the returns perhaps overestimated?
- How can we rapidly convert zero-return AI investments into tangible value through quick wins?
- In what ways can industry, HTA bodies, and researchers collaborate to unlock the full potential of AI in healthcare?
- When planning an AI study - such as using Natural Language Processing (NLP) with digital listening to map the patient journey and generate epidemiology data - what best practices ensure that the methodology is scientifically robust and likely to be accepted as valid evidence by journals, regulators, HTA bodies, or payers? Could you share insights into essential steps, validation frameworks, or stakeholder engagement strategies to maximise credibility and acceptance?
- With the emphasis of human-in-the-loop, could the panellists speak to Julian's point about the challenge of closing the loop in healthcare-related questions? Is expert validation of AI outputs sufficient? Is there work towards closing the loop in ways that are grounded in the unknowns of the diseases?
- We spoke about alignment across organisations and collaboration. What are the platforms and forums to facilitate and foster discussion and pilot projects? Is there a risk that we are continuing to work in silos, and perhaps not moving as quickly as the technology is advancing?
- What can we do in the general application of AI to improve our evidence generation to consider unknown biases?
- What are the areas of collaboration where it would really be fruitful to bring the AI uses forward? What stakeholders should be involved in this collaboration?

Several important learnings followed from this conversation. Firstly, panellists broadly agreed that **AI should be used to augment and not replace human expertise**. It is essential for all stakeholders to understand and get prepared to the use of AI, but it is equally essential to keep human validation and oversight, particularly in healthcare where decisions have significant consequences.

Secondly, the discussion highlighted the value of **starting with low-risk but high-impact use cases**. Speakers provided examples of rapid AI investments through quick wins, including using AI for systematic literature reviews, plain language translation of HTA documents, and capturing patient experience data through AI - as recommended by the European Medicines Agency (EMA). These applications were seen as practical entry points for building trust and demonstrating value.

NICE and CDA-AMC stressed the importance of **transparent validation studies**, especially when AI-generated evidence plays a pivotal role. Stratified performance metrics were recommended to assess fairness and bias of assessment algorithms across subpopulations.

Panellists also emphasised the need for **collaboration across stakeholders** (between HTA bodies, but also with patients, industry, and AI specialists) to ensure ethical, inclusive, and effective AI adoption through mutual learnings. Speakers identified systematic literature

reviews as a key promising area of collaboration. They emphasised the high potential of unstructured data to improve understanding of real-world effectiveness and safety, particularly in post-HTA contexts.

Final conclusions focused on what panellists wanted the audience to remember and act on, leading to the following **takeaways**:

- AI is already influencing the evidence generation pipeline, but its adoption across HTA bodies and industry remains at early stages.
- AI should augment but not replace human judgement. Across all interventions, speakers emphasised the importance of human oversight, especially in high-risk healthcare contexts. In this context, terminology matters and speakers mentioned that augmented (rather than artificial) intelligence better captures the role of AI in supporting (not replacing) human expertise.
- Panellists called for early and ongoing collaboration and dialogue between HTA bodies, patients, AI specialists, and patients to build trust and encourage ethical AI adoption. This stakeholder engagement should acceleration the "learning by doing" process and build confidence and trust in AI-enabled evidence generation.

EXTENDING THE DIALOGUE: ANSWERS TO OPEN Q&A

- *What is the first area of investment for companies to start building AI capacities: workforce training or technical capabilities in terms of tools and AI solutions?*

Answer: These two areas should not be mutually exclusive, actually these should go hand in hand in order to improve the adoption level. As shown in the AI adoption maturity framework, in order to advance into the next level of maturity in adoption, it requires investment in the AI tools and solutions embedded into workflow, improve workforce skills, governance, as well as the impacts on decision making. When making investment decisions, leaders need to ensure these key areas are developing in sync, not in an isolated approach.

- *You classified using AI in economic models as high risk. Recently, NICE HTA Lab published a report about this particular use. Do you agree with their recommendations?*

Answer: Yes, I agree with the recommendations recently published in the NICE HTA Innovation Laboratory (HTA lab) Report around 'Generative artificial intelligence (AI) in health economic modelling'. While it is encouraging to see that NICE has spearheaded in exploring use cases and proposing principles that will hopefully translate into HTA technical guidance for AI in Health Economic Evaluation in the near future, the main challenges identified in this report, such as accuracy and reliability, data privacy and security, lack of technical expertise, and limited guidance from national bodies, will take some time to resolve. Given the impact of the reimbursement and access decisions based on these Health Economic Models and Evaluations, and the uncertainty and assumptions used in these analyses, I think this area remains a high-risk area for the time-being until the above-mentioned challenges can be addressed

- *Related to the question about HTA, have you seen AI incorporated in health economic models?*

Answer: At NICE, we have explored the use of generative AI with economic modelling as part of our HTA lab, you can read the published results on the NICE website here. While demonstrations and stakeholder feedback have been sought on this topic as part of the lab, the next stage would be to undertake some validation for potential economic model use cases (perhaps less risky ones to begin with, alongside but not replacing normal processes) to ensure that this could be conducted in a robust and transparent way.

- *Clearly, many components of a HTA submission (to TOLV, NICE, CDA-AMC, or even JCA) can be done with gen AI. Even the dossier itself. As long as the developers take ownership of the contents, why would HTA organisations care how it is developed (and how would they know)?*

Answer: I actually don't think it's currently 'clear' how much can be trusted to generative AI. One of the reasons NICE is asking companies to declare where AI has been used in their dossier submissions is so that we can monitor whether this is being done in a safe, transparent and thoughtful manner as well as to help us understand how central to the dossier submissions AI tools are becoming.

RWE4Decisions REAL WORLD EVIDENCE

RWE4Decisions is a payer-led multi-stakeholder learning network, which has developed **stakeholder actions** that will better enable the use of real-world evidence in HTA/payer decisions about highly innovative technologies.

The work has been commissioned by the Belgian National Institute of Health and Disability Insurance (NIHDI) and is led by a multistakeholder **Steering Group** with a wider community of contributors including HTA bodies and payers, regulatory agencies, patient groups, clinicians, industry, analytics experts and academic experts/researchers.

The RWE4Decisions Secretariat is provided by FIPRA, with sponsorship in 2025 by the European Confederation of Pharmaceutical Entrepreneurs (EUCOPE), Astellas, AstraZeneca, Gilead, MSD, Roche and Takeda.

For further information and to watch the recording of the webinar, visit our **website**.

What are you are doing to progress learnings on the use of RWE?

Contact us at **secretariat@rwe4decisions.com** to join the RWE4Decisions Learning Network.

Follow us for more!

@RWE4Decisions