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Transforming Modern Healthcare: From Real-World Data to Trusted Real World Evidence

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Abstract

Healthcare is undergoing a profound transformation driven by the digitalisation of healthcare systems, the availability of real-world data (RWD), and advances in artificial intelligence (AI). While randomized controlled trials (RCTs) remain the cornerstone of evidence-based medicine, they are not designed to answer every question encountered in routine clinical practice, particularly among diverse patient populations with multimorbidity, polypharmacy, and complex healthcare needs. Consequently, real-world evidence (RWE), generated through the rigorous analysis of healthcare data, has emerged as an essential complement to evidence from RCTs. This editorial discusses the evolving role of RWE in supporting clinical practice, regulatory decision-making, health technology assessment, and healthcare policy. The availability of electronic health records, disease registries, administrative databases, pharmacy records, and digital health resources is creating unprecedented opportunities to understand treatment effectiveness, medication safety, healthcare utilisation, and patient outcomes in routine care. It further examines the transformative role of AI in accelerating data curation, extraction, and analysis, while emphasising that technological advances cannot substitute for rigorous scientific methodology. Robust pharmacoepidemiological principles, including rigorous study design, appropriate analytical methods, transparency, and reproducibility, remain fundamental to generating trustworthy evidence from complex healthcare data. Finally, the editorial discusses governance, multidisciplinary collaboration, workforce development, and responsible innovation in building sustainable RWE ecosystems. As healthcare continues to evolve, the future of evidence generation will depend not on the volume of data or the sophistication of compuxtational tools alone, but on the ability to transform RWD into credible evidence that improves patient outcomes, informs healthcare decisions, and strengthens public trust.

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Introduction

For more than half a century, randomized controlled trials (RCTs) have been the cornerstone of evidence-based medicine. Their rigorous design, characterised by randomisation, carefully defined eligibility criteria, and controlled study environments, has revolutionised healthcare by providing robust evidence on the efficacy and safety of medicines, medical devices, and other healthcare interventions. The ability of RCTs to minimise bias and establish causal relationships has rightly positioned them as the gold standard for regulatory approval and clinical guideline development (Granholm et al., 2022). Their contribution to modern medicine is unquestionable and remains indispensable.

However, healthcare has changed considerably since the evidence hierarchy was first established. Ageing populations, rising multimorbidity, polypharmacy, and increasingly diverse patterns of healthcare delivery have created clinical questions that extend beyond the scope of traditional clinical trials. While RCTs remain essential for determining whether an intervention can work under carefully controlled conditions, healthcare decision-makers increasingly need to know whether it works in routine clinical practice and whether it remains safe and effective across the diverse populations encountered in everyday care. Older adults, individuals living with multiple chronic conditions, pregnant women, patients with chronic kidney disease, and those receiving complex medication regimens frequently represent the reality of clinical practice, yet their experiences are often underrepresented or excluded from traditional RCTs (Lambourg, 2024).

In parallel with these changing evidence needs,the digital transformation of healthcare has created unprecedented opportunities to address these evidence gaps. Advances in biomedical science, digital technologies, and increasingly interconnected healthcare systems have fundamentally changed not only how healthcare is delivered, but also how knowledge is generated to support clinical practice, regulatory decision-making, and health policy (Tang et al., 2024). Every patient encounter, prescription, laboratory result, diagnostic image, and clinical outcome now contributes to an expanding digital footprint that reflects the realities of healthcare as it is delivered every day (Stoumpos et al., 2023). This unprecedented availability of real-world health data, commonly referred to as real-world data (RWD), offers an extraordinary opportunity to deepen our understanding of disease, optimise treatment strategies, improve patient safety, and ultimately transform healthcare into a learning health system.

The emergence of large-scale RWD has made it increasingly possible to address many clinically important questions that are difficult, and in many cases impractical, to answer through RCTs. These include evaluating long-term treatment effectiveness, medication adherence, healthcare utilisation, comparative effectiveness, prescribing patterns, healthcare inequalities, and rare adverse events across large, diverse, and heterogeneous patient populations over extended periods of follow-up (Gale et al., 2023). For pharmacists, RWD provides valuable insights into medication utilisation, medication adherence, polypharmacy, medication safety, and the effectiveness of pharmaceutical care interventions in routine clinical practice, thereby supporting evidence-based medication management and optimising patient outcomes. The widespread adoption of electronic health records, disease registries, pharmacy dispensing databases, administrative claims, laboratory information systems, imaging repositories, patient-reported outcome measures, wearable devices, and digital health applications has transformed healthcare into one of the world's richest sources of continuously generated information (Liu & Panagiotakos, 2023). Never before has it been possible to observe healthcare delivery across entire populations with such breadth, depth, and temporal continuity. As healthcare systems become increasingly digital and interconnected, the scale, richness, and longitudinal depth of RWD continue to expand, providing unprecedented opportunities to better understand disease patterns, treatment pathways, and

healthcare delivery in routine clinical practice.

When RWD are analysed using rigorous scientific methods and robust study designs, they generate real-world evidence (RWE) (Wilson & Booth, 2024). Together, RCTs and RWE provide complementary perspectives: one establishing whether an intervention has the potential to work, the other demonstrating how it performs in the settings where patients receive care. Collectively, they offer a more complete evidence base for improving clinical decision-making, informing healthcare policy, and increasingly supporting regulatory decision-making alongside evidence from traditional RCTs (Gomes et al., 2022; *U.S.* Food and Drug Administration, 2018). Increasingly, regulatory agencies, health technology assessment bodies, and clinical guideline developers recognise RWE as an important complement to evidence generated from RCTs.

Adding further momentum to this transformation, artificial intelligence (AI), including machine learning, natural language processing, and large language models, is reshaping healthcare research. They are rapidly changing how healthcare data are processed, curated, and analysed. Tasks that once required months of painstaking manual effort, including extracting medication information from unstructured clinical records, harmonising heterogeneous datasets, identifying complex disease phenotypes, and integrating multiple data sources, can now increasingly be performed with greater speed and efficiency (Nerella et al., 2024). These advances have the potential to accelerate every stage of the RWE pipeline, from data preparation to evidence generation.

Perhaps the greatest contribution of AI lies not simply in analysing larger volumes of data, but in unlocking information that has historically remained inaccessible. Much of the clinically relevant information within healthcare systems exists as free-text clinical notes, discharge summaries, pathology reports, imaging interpretations, and prescription instructions. Advances in natural language processing and large language models are enabling researchers to transform these previously underutilised sources into structured, analysable data, thereby expanding both the scope and richness of RWD. Moreover, by converting vast amounts of previously inaccessible unstructured information into analysable data, AI is substantially increasing the volume, diversity, and complexity of RWD available for research. These advances create unprecedented opportunities for generating richer and more comprehensive RWE.

However, despite these technological advances, RWE is not without its challenges. The growing capabilities of AI should not distract us from a fundamental scientific principle; algorithms do not replace methodology. AI may identify patterns within data, but it cannot determine whether a research question is clinically meaningful, whether an appropriate comparator has been selected, whether a causal relationship truly exists, or whether observed associations are the consequence of systematic bias. These remain scientific judgements that require domain expertise, methodological rigour, and critical interpretation. The quality of RWE is fundamentally determined by the quality of the underlying data and the rigour of the methods used to analyse them.

The availability of large volumes of healthcare data should not be mistaken for the availability of reliable evidence. RWD are collected primarily to support clinical care and healthcare administration rather than research. Consequently, they are inherently complex, frequently fragmented across multiple healthcare settings, and often affected by missing information, inconsistent coding, measurement error, and systematic bias (Al-Sahab et al., 2024). Large datasets may increase statistical precision, but they do not automatically improve scientific validity. Without rigorous study design, careful analytical methods, and thoughtful interpretation, more data may simply generate more uncertainty rather than more knowledge. Likewise, increasingly sophisticated analytical tools cannot compensate for weaknesses in study design. Selection bias, confounding by indication, immortal time bias, exposure misclassification, missing data, and measurement error remain fundamental challenges in observational research (Acton et al., 2023). Left unaddressed, these methodological limitations can produce highly precise estimates

that are nevertheless misleading.

Taken together, these challenges underscore a broader reality: the future of healthcare depends not simply on our ability to collect and analyse ever-growing volumes of data, but on our ability to transform those data into evidence that is scientifically robust, clinically meaningful, and trusted by patients, clinicians, regulators, and policymakers. This transformation represents both one of the defining challenges and one of the greatest opportunities in contemporary healthcare research. It also raises a fundamental question: how can increasingly complex RWD be transformed into evidence that is both scientifically credible and capable of improving patient outcomes? The answer lies in recognising that healthcare data, regardless of their size or complexity, do not generate evidence on their own. Evidence emerges only when data are interpreted through rigorous scientific methods. At the heart of this transformation lies pharmacoepidemiology, the discipline that provides the methodological foundation for generating trustworthy RWE capable of informing clinical practice, regulatory decision-making, and health policy.

Pharmacoepidemiology is far more than the statistical analysis of large healthcare databases. Grounded in the principles of epidemiology, clinical pharmacology, and biostatistics, it provides the conceptual and methodological framework needed to formulate clinically relevant research questions, define appropriate study populations and comparators, determine treatment initiation and follow-up, accurately measure exposures and outcomes, and address the many sources of bias inherent in observational research. These methods are particularly valuable in pharmacy practice, where RWE can inform medication optimisation, evaluate the safety of complex drug regimens, monitor adverse drug reactions, assess medication adherence, and evaluate pharmaceutical care interventions, thereby supporting evidence-based medication management and improving patient outcomes. These principles are further reinforced through internationally recognised Good Pharmacoepidemiology Practices, which promote transparency, methodological rigour, and reproducibility throughout the research process (ISPE Commentary, 2008).

Each methodological decision influences whether study findings reflect true treatment effects or systematic error. Over the past two decades, remarkable advances in pharmacoepidemiological methods have further strengthened the credibility of RWE. Approaches such as propensity score methods, target trial emulation, causal inference frameworks, quantitative bias analysis, active comparator new-user designs and advanced sensitivity analyses have enabled researchers to more closely emulate the conditions of RCTs while preserving the strengths of observational data. Collectively, these methodological innovations have substantially enhanced confidence in RWE and contributed to its growing acceptance by regulatory agencies, health technology assessment bodies, clinicians, and policymakers worldwide. This growing acceptance is reflected in dedicated RWE frameworks developed by organisations such as NICE to support evidence generation for health technology assessment (National Institute for Health and Care Excellence, 2022).

Equally important is the responsible stewardship of patient data. As RWE increasingly relies on electronic health records, administrative databases, and other routinely collected healthcare data, protecting patient privacy, ensuring robust data security, and maintaining compliance with ethical and legal frameworks are essential for sustaining public trust. Responsible data governance, including appropriate data de-identification, secure data access, and transparent data use, must remain central to the development of AI-enabled RWE.

Beyond data governance, the integration of AI into healthcare research also brings broader scientific and ethical responsibilities. Its credibility will ultimately be determined not only by computational performance, but also by adherence to the scientific and ethical principles that underpin responsible research. As international reporting and governance standards continue to evolve, adherence to these frameworks will be essential to ensure that AI-enabled healthcare research remains

transparent, reproducible, accountable, and worthy of public trust (Collins et al., 2024). Technological innovation and scientific integrity must therefore advance together. AI should be viewed not as a replacement for pharmacoepidemiology, but as a powerful partner that enhances scientific discovery while remaining firmly grounded in established epidemiological principles.

For countries investing in digital health transformation, this convergence of robust methodology and technological innovation presents an unprecedented opportunity. Nations that combine high-quality healthcare data, strong governance frameworks, methodological expertise, and responsible AI implementation will be well positioned to build trusted RWE ecosystems capable of informing healthcare decisions at both national and international levels. International initiatives such as the European Medicines Agency's DARWIN EU programme illustrate how coordinated national and regional RWE infrastructures can support regulatory decision-making, post-marketing surveillance, and evidence-informed healthcare policy (European Medicines Agency, 2026).

Malaysia is well positioned to strengthen its RWE ecosystem through continued investment in digital health, healthcare data infrastructure, and methodological expertise. The expansion of electronic health records, national disease registries, administrative healthcare databases, and health technology assessment capabilities provides a strong foundation for generating high-quality RWE. Realising this potential, however, will require addressing persistent challenges, including fragmented data systems, limited interoperability, evolving governance frameworks, workforce capacity, and sustained investment in data quality. Overcoming these challenges will be essential to ensuring that healthcare data are transformed into trusted evidence capable of informing clinical practice, regulatory decision-making, and health policy.

Developing national expertise in pharmacoepidemiology, biostatistics, clinical informatics, data science, and artificial intelligence will be fundamental to building a sustainable RWE ecosystem and ensuring that high-quality evidence informs healthcare decision-making. Beyond strengthening its national capacity, Malaysia also has the opportunity not merely to adopt international best practices, but to contribute to shaping the future of RWE within the Asia-Pacific region by demonstrating how scientific excellence, responsible innovation, and strategic collaboration can strengthen healthcare systems and improve population health. Ultimately, the true value of RWE lies not in the volume of data analysed or the sophistication of analytical methods, but in its ability to improve patient outcomes, enhance healthcare quality, reduce health inequities, and support better healthcare decisions.

Achieving this vision, however, will require far more than technological advancement or methodological innovation. The future of RWE depends upon a shared commitment to collaboration across the entire healthcare ecosystem. Universities, healthcare providers, policymakers, regulatory authorities, health technology assessment agencies, professional societies, industry partners, and patients each bring unique perspectives and expertise that are essential for building a trusted and sustainable RWE ecosystem. Among these stakeholders, pharmacists play a pivotal role through medication stewardship, pharmacovigilance, optimisation of pharmacotherapy, and the generation, interpretation, and translation of RWE into clinical practice. Positioned at the interface between medicines, patients, and healthcare systems, pharmacists are uniquely equipped to contribute to evidence generation while applying RWE to optimise medication use and improve patient outcomes. No single organisation or discipline can realise the full potential of RWE in isolation. Meaningful collaboration extends well beyond data sharing. It requires the development of common data standards, interoperable healthcare systems, transparent governance frameworks, methodological excellence, and a culture that values openness, reproducibility, and continuous learning. Equally important is meaningful patient engagement. As the ultimate beneficiaries of healthcare research, patients should not simply

contribute data but also help shape the research questions, outcomes, and priorities that define future evidence generation. Only through genuine partnership can RWE remain scientifically robust, clinically relevant, and socially accountable.

The future of healthcare will be shaped not by the quantity of data we collect, nor by the sophistication of the technologies we develop, but by our ability to transform information into trusted knowledge and trusted knowledge into better decisions. The greatest promise of RWE is not simply that it enables us to study healthcare as it exists today, but that it empowers us to improve the healthcare of tomorrow. This future will not emerge automatically. It must be built through scientific excellence, responsible innovation, ethical stewardship, and sustained collaboration across disciplines, institutions, and nations. As healthcare continues to evolve, RWE should no longer be regarded as a complementary source of information, but as an essential pillar of evidence-informed healthcare, one that bridges research and practice, connects discovery with implementation, and transforms routine healthcare experiences into knowledge that benefits future patients.

Conclusion

The journey from data to decisions is ultimately a journey of trust. Trust in the quality of our data. Trust in the rigour of our scientific methods. Trust in the integrity of our technologies. In this context, trustworthiness is built through transparent methods, high-quality and secure data, rigorous scientific standards, reproducible findings, and ethical governance that collectively ensure evidence is reliable, accountable, and fit for healthcare decision-making. And above all, trust that the evidence we generate will improve the health and well-being of the people we serve. If we remain committed to these principles, RWE will become far more than another research methodology. It will serve as the engine of a continuously learning healthcare system, one capable of delivering safer, more effective, more equitable, and more patient-centred care for generations to come.

Declaration of generative AI and AI-assisted technologies in the writing process

During the preparation of this manuscript, generative AI tools (e.g., ChatGPT, Gemini, and similar technologies) were used solely to enhance the language, grammar, readability, and overall clarity of the writing. All scientific content was written, critically reviewed, verified, and approved by the authors, who take full responsibility for the accuracy, integrity, and originality of the manuscript

References

Acton, E., AW Willis, & Hennessy S. (2023). Core concepts in pharmacoepidemiology: Key biases arising in pharmacoepidemiologic studies. *Pharmacoepidemiol Drug Saf., 32*(1).

Al-Sahab, B., Leviton, A., Loddenkemper, T., Paneth, N., & Zhang, B. (2024). Biases in Electronic Health Records Data for Generating Real-World Evidence: An Overview. *Journal of Healthcare Informatics Research, 8*(1), 121–139. https://doi.org/10.1007/s41666-023-00153-2

Collins, G. S., Moons, K. G. M., Dhiman, P., Riley, R. D., Beam, A. L., Van Calster, B., Ghassemi, M., Liu, X., Reitsma, J. B., van Smeden, M., Boulesteix, A.-L., Camaradou, J. C., Celi, L. A., Denaxas, S., Denniston, A. K., Glocker, B., Golub, R. M., Harvey, H., Heinze, G., . . . Logullo, P. (2024). TRIPOD+AI statement: Updated guidance for reporting clinical prediction models that use regression or machine learning methods. *BMJ, 385*, e078378. https://doi.org/10.1136/bmj-2023-078378

European Medicines Agency. Data Analysis and Real World Interrogation Network (DARWIN EU). (May 20, 2026). Retrieved July 21, 2026, from https://www.ema.europa.eu/en/about-us/how-we-work/data-regulation-big-

data-other-sources/real-world-evidence/data-analysis-real-world-interrogation-network-darwin-eu

Gale, R. P., Zhang, M.-J., & Lazarus, H. M. (2023). The role of randomized controlled trials, registries, observational databases in evaluating new interventions. *Best Practice & Research Clinical Haematology*, *36*(4), Article 101523. https://doi.org/10.1016/j.beha.2023.101523

Gomes, M., Latimer, N., Soares, M., Dias, S., Baio, G., Freemantle, N., Dawoud, D., Wailoo, A., & Grieve, R. (2022). Target trial emulation for transparent and robust estimation of treatment effects for health technology assessment using real-world data : opportunities and challenges. *Pharmacoeconomics*, *40*(6), 577–586. https://doi.org/10.1007/s40273-022-01141-x

Granholm, A., Alhazzani, W., Derde, L. P. G., Angus, D. C., Zampieri, F. G., Hammond, N. E., Sweeney, R. Mac, Myatra, S. N., Azoulay, E., Rowan, K., Young, P. J., Perner, A., & Møller, M. H. (2022). Randomised clinical trials in critical care: past, present and future. *Intensive Care Medicine*, *48*(2), 164–178. https://doi.org/10.1007/s00134-021-06587-9

ISPE Commentary. (2008). Guidelines for good pharmacoepidemiology practices (GPP). *Pharmacoepidemiology and Drug Safety*, *17*(2), 200-208. https://doi.org/10.1002/pds

Lambourg, E. (2024). Improving the quality of pharmacoepidemiological studies using the target trial emulation framework. *Nature Reviews Nephrology*, *20*(769), https://doi.org/10.1038/s41581-024-00884-4

Liu, F., & Panagiotakos, D. (2022). Real-world data: a brief review of the methods, applications, challenges and opportunities. *BMC Medical Research Methodology*, *22*(1), 287. https://doi.org/10.1186/s12874-022-01768-6

National Institute for Health and Care Excellence. (2022). *NICE real-world evidence framework*.

Nerella, S., Bandyopadhyay, S., Zhang, J., Contreras, M., Siegel, S., Bumin, A., Silva, B., Sena, J., Shickel, B., Bihorac, A., Khezeli, K., & Rashidi, P. (2024). Transformers and large language models in healthcare: A review. *Artificial Intelligence in Medicine*, 154, Article 102900. https://doi.org/10.1016/j.artmed.2024.102900

Stoumpos, A. I., Kitsios, F., & Talias, M. A. (2023). Digital transformation in healthcare: Technology acceptance and its applications. *International Journal of Environmental Research and Public Health*, 20(4), Article 3407. https://doi.org/10.3390/ijerph20043407

Tang, A. S., Woldemariam, S. R., Miramontes, S., Norgeot, B., Oskotsky, T. T., & Sirota, M. (2024). Harnessing EHR data for health research. Nature medicine, 30(7), 1847-1855. https://doi.org/10.1038/s41591-024-03074-8

U.S. Food and Drug Administration. (2018, December). *Framework for FDA's real-world evidence program*. https://www.fda.gov/media/120060/download

Wilson, B. E., & Booth, C. M. (2024). Real-world data: Bridging the gap between clinical trials and practice. *eClinicalMedicine, 78*, Article 102915. https://doi.org/10.1016/j.eclinm.2024.102915