

What to Expect in 2024: Important Health Economics and Outcomes Research (HEOR) Trends

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In this paper—being published concurrently in ClinicoEconomics and Outcomes Research, Current Medical Research and Opinion, Expert Review in Pharmacoeconomics and Outcomes Research, Journal of Medical Economics and—we, as the Editors-in-Chief of the journals, want to share our perspectives on the emerging trends in health economics and outcomes research (HEOR) for 2024. Our intent is not to set an editorial agenda but to highlight what we think are topics of key importance.

We hope this article shows you—the journals' readers and authors—our commitment to keeping these journals at the forefront of research and scholarship in clinical outcomes, effectiveness, value, and economics. We aim for a forward-looking, global, and equitable approach to content that balances innovation with replication, validation and application. Our objective is to advance these interlocking fields and translate new knowledge into practical and actionable information.

As editors, we are dedicated to using our journals as scientifically grounded, peer-reviewed channels to enable cost-responsible, equitable, and quality healthcare, turning limited availability into fair accessibility for those in local, regional, and global settings.

Herein, each Editor-in-Chief contributes their personal perspectives on trends in HEOR for 2024 and how building on these trends in the coming year will be critical to further innovation.

I. Alternate and Novel Metrics of Benefit and Harm

In the US, the 2022 Inflation Reduction Act (IRA) enables Medicare – the federal health insurance for people aged 65 or older, younger people with disabilities, and people with end-stage renal disease – to negotiate drug prices. However, as Shafrin and colleagues note,¹ the use of “conventional methods of cost-effectiveness analysis that treat life-years gained as less valuable when they accrue in sicker and more disabled patients” is prohibited. This means that, in particular, the standard unadjusted quality-adjusted life year (QALY) is off-limits. Without wading into the scientific, political, and polemic debates, the issue is a much-needed reminder that the field of health economics needs to expand its library of indicators with alternate if not novel metrics of the value of treatments. Shafrin et al offer the equal value of life-year gained (evLYG) measure, a metric also used by the Boston-based Institute for Clinical and Economic Review. This metric “evenly measures any gains in length of life, regardless of the treatment’s ability to improve patients’ quality of life” as an alternative to valuing life extension, and can be complemented by the Generalized Risk-Adjusted Cost-Effectiveness (GRACE) methodology.^{2–4}

The ripple effect of the IRA goes well beyond the US. In fact, it offers the field of health economics an opportunity to go wider – and in doing so, to enrich the repertoire of metrics available for valuing treatments. The metrics cited above, valuable as they are in their own way, are (still) focused on survival. Not every treatment – medical, pharmaceutical, surgical, or otherwise interventional – aims to extend life and may instead target shorter term and different outcomes.

Graphical Abstract



The field needs more and broader metrics of benefit, that is, intended desirable outcomes. Just as much, it needs metrics that accommodate harm: less of an undesirable outcome, whether intended or unintended. Consider, for instance, adverse treatment effects, which we can assume are unintended (but therefore not necessarily unanticipated). For instance, the QALY method “penalizes” the already imperfect health state of an illness with a utility of less than 1.0, from which additional disutilities are subtracted as the disease worsens despite treatment but also as patients experience various grade III/IV adverse effects. Economic evaluations of benefits gained will be more holistic if complemented by separate economic evaluations of harms averted. Not only, how much more (or how much less) does an intervention cost to gain (or lose) a unit of benefit; but also, how much more (or how much less) does the same or a complementary intervention cost to avert (or fail to avert) a unit of harm? In fact, some economic evaluations of treatments may have harm reduction as the primary intended outcome. This calls for metrics in which the unit of outcome is expressed in clinical units.

The focus on life extension will (and should) continue to prevail when a treatment is inherently life-extending, and the underlying illness is inherently life-shortening. However, some treatments address important short-term problems; for instance, anticoagulants are used to lower the risk of acute deep venous thrombosis after major abdominal surgery. Treatments may target a problem where the intended outcome is short-term; for instance, lowering the risk of chemotherapy-induced neutropenia in one cycle of treatment by prophylacting with granulocyte colony-stimulating factors. The treatment may be surgical; for instance, minimally invasive lumbar spine surgery where the benefits gained are critical (improved or restored mobility and function in less time, decreased pain, better quality of life, ...) yet also the harms averted (wound complications, hospitalization, delayed mobility, and function, ...). True, there may always be a risk of death, but is the death attributable to a failed treatment or a severe adverse event within a clinically reasonable time? This too calls for metrics based on clinical units.

2. Patient Preferences

Interest in patient preference research to assess health technologies and inform payer decisions has drastically increased.⁵ Patient preference studies have used qualitative or quantitative techniques to investigate the relative importance of outcomes or other attributes that characterize a health intervention or situation. In particular, the use of discrete-choice experiments (DCEs) is nowadays increasingly being used to evaluate patient preferences through statistical analysis. Several HTA organizations have recently included patient preference information in their decision-making guidelines.⁶

In recent years, several patient preference research papers have been published in our journals. Some examples include, Shiozawa et al who assessed the preferences for vasomotor symptoms (VMS) in US women, and revealed that sleep improvement and reductions in VMS frequency and severity were the most important treatment attributes.⁷ In another study, Boger et al revealed the preference of young adults for COVID-19 vaccination in the United Kingdom and identified some differences in the importance of attributes among the participants using latent class and subgroup analyses.⁸ Willems et al also used a DCE to elicit the treatment preferences of patients with hidradenitis suppurativa in the US and revealed that patients considered effectiveness and pain reduction to be the most important treatment characteristics.⁹

With the growing interest and importance given to patient preference research, we could expect an increasing number of studies in various health care contexts, for example to assess trade-offs between health outcomes or treatment characteristics, to value experienced factors or to develop priority-setting framework. Furthermore, research summarizing current evidence about patients' preferences in various fields would be of interest, and efforts to increase their comparability and standardization are projected. Furthermore, additional guidelines on the conduct and appraisal of these studies as well as on their use in healthcare decision-making would be worthwhile.

3. Real-World Evidence

The US FDA defines Real-World Evidence (RWE) as clinical evidence about the usage and potential benefits or risks of a medical product derived from analysis of real-world data (RWD). RWD are data relating to patient health status routinely collected from a variety of sources such as electronic health records, medical claims data, data from product or disease registries, and data gathered from other sources (such as digital health technologies) that can inform on health status.¹⁰ In addition, artificial intelligence (AI) solutions have been authorized for use to improve automation and learning of medical devices,¹¹ the efficiency of diagnostic/therapeutic development and commercial manufacturing, regulatory assessment, and post-market surveillance, among many other potential applications.¹⁰

In recent years, several developments and initiatives have contributed to fostering interest in RWE use for healthcare decision-making.¹² This mainly arises from the opinion that the current decision-making process is largely relying on evidence generated from randomized clinical trials (RCT). However, it is well understood that the ultimate decision can be affected by an array of factors (Figure 1).¹³

Under this environment, RWE is well positioned to serve as a complimentary source of evidence leading to a higher external validity of RCTs. Good examples of real-world data that are not captured by RCTs but can have significant impact on clinical and economic outcomes include just to name a few: patient compliance, long-term toxicity, and long-term effects on quality of life of healthcare technologies.

We are in the 21st century and with the availability of a variety of electronic devices, researchers are tasked with the responsibility to generate high quality, transparent, representative, and replicable data to assist better-informed decision-making.

However, as we are steaming ahead in this direction, we should also bear in mind that there are always less privileged groups who would need support due to unaffordability and poor health literacy. This will require a collaborative effort between government, researchers, and industry to achieve the desirable health outcomes.

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Figure 1 Drivers of healthcare decision-making process.

4. Equity

The World Health Organisation (WHO) defines equity as “the absence of avoidable or remediable differences among groups of people, whether those groups are defined socially, economically, demographically, or geographically”.¹⁴ When used in healthcare, equity in health refers to the fairness in the distribution of health across individuals. It may also refer to the distribution of health care (for example, expenditure, utilisation, or access to care). Any inequalities will lead to significant consequences on health and social harmony.

In recent years, many healthcare plans have adopted policies to control medication costs. These measures include increasing beneficiary co-payments, mandating the use of generic drugs, requiring mail-order services, and expanding formulary use. These policies have significant impacts on overall drug expenses. Such substantial changes often raise concerns about potential negative health outcomes, especially for individuals with chronic illnesses.¹⁵ Indeed, major shifts in drug benefits are at times linked to significant morbidity and mortality in specific high-risk population. In certain cases, these changes might even prompt enrollees to cease their therapy. Adjustments in copayments can markedly impact enrollees’ out-of-pocket expenses, the persistence of medication usage, and potentially the quality of healthcare.¹⁶

The COVID-19 pandemic has suggested that a reliance on clinical trials alone in assessments may delay access to novel, innovative health technologies.¹⁷ The pandemic has further exposed an inequity in accessing healthcare among different populations, which has led to health outcome disparities among groups from different socioeconomic backgrounds.

Currently, health economic assessment conclusions on the benefits measured by cost-effectiveness analysis (CEA) and cost-utility analysis (CUA) are usually based on the extra-welfarist approach where benefits accruing to any individual are assumed to be worth the same to others in the society. This assumption is obviously an oversimplification of the real-world environment. As a result, researchers have started to extend the CEA and CUA to include other population groups by using Extended CEA and Distributional CEA to achieve equity in deciding benefit distribution.¹⁸ Other papers have suggested to adopt an approach where cost-benefit analysis (CBA) informed by the willingness-to-pay (WTP) from diverse backgrounds and supplemented by health-related quality-of-life (HRQoL) from within and between populations will provide better-informed decision-making.¹⁹

Looking ahead, the research community should consider if an extension of CEA, CUA and CBA should be made a requirement for achieving equity in their research.

5. Access and Equity to Therapeutics in Low- and Middle-Income Countries

The global community now acknowledges that assuring access to medicines, and certainly essential medicines, in low- and middle-income countries (LMICs) is in part the responsibility and in part the duty of high-income countries (HICs).^{20,21} Access and equity to therapeutics in LMICs are (edging towards) common parlance yet not necessarily common action, and health and pharmacoeconomic research and innovation is needed to further this cause. As Daems

elaborated so well in his book on medicines for the developing world, there is a multifactorial context that needs to be considered and studied further – each factor separately and many of these factors at their confluence: economic status and poverty reduction; risk management, risk sharing and investment incentives; intellectual property and licensing; pricing within and across jurisdictions; production; procurement and availability; international trade; capacity building, from infrastructure to people; availability, quality, and safety; and global public health policy.²² All this touches upon other issues in need of further investigation: affordability; health care financing; regulatory challenges; and addressing regionally endemic diseases.

Biosimilars will prove to be key to bringing biological treatments from their prevailing markets of 1 billion people in HICs to the remaining 7 billion in LMICs.²³ On the face of it, the economics of commodities, which is what biosimilars are, may seem less exciting than the economics of innovations. However, they are an essential area of inquiry to bring biological treatments to LMICs, as a cost-efficiency and expanded access study on biosimilar rituximab by Halawah and colleagues demonstrated for Jordan.²⁴ With the proliferation of biosimilar versions of singular reference products in the biosimilar HIC markets, the survival of several biosimilar manufacturers in HICs will depend on their willingness and ability to reach out to LMICs – either independently or through regional or local partnerships. Add to this the nascent biosimilar industries in several middle-income countries, which need to be supported in their market access in both the short- and the long-term. With supporting research and innovation, the benefits are major: cost-efficiencies from savings to expanded access to treatment on a budget-neutral basis; market competition with reference products but also with other biosimilars, driving down prices and increasing affordability; and a broader epidemiological and therapeutic reach – in addition to creating jobs in LMICs.

6. Economics of Prevention

With the increasing burden of chronic diseases such as heart disease, diabetes, and cancer, there is a growing emphasis for people of all ages on preventive measures such as healthy eating, regular exercise, reduced alcohol or smoking consumption, or stress reduction. Many preventive measures may generate substantial economic benefits by lowering healthcare costs, but also through broader economic and societal benefits. Assessing the economic value and health consequences of prevention is becoming crucial to support their value and convince policy makers about their importance, and therefore to pave the way for more preventive measures in our healthcare systems.

Some cost-effectiveness analyses of preventive interventions have already been published. For example, the study by Oh et al suggested that the universal testing for BRCA status of all US women at age 40 provides short-term and long-term economic value using a decision-analytic model.²⁵ In another economic study, Diakite et al found that switching from bivalent to nonvalent HPV vaccination would be considered cost-effective in Norway.²⁶ Other types of health economics studies were performed in the field of prevention including a study by Al-Omar HA et al that estimated the cost-consequence analysis of weight loss on obesity-related outcomes in privately insured adults with obesity in Saudi Arabia and suggested that for a 15% weight loss, 18.8% of incidence cases of obesity-related outcomes may be prevented.²⁷

We are expecting an increasing number of health economics studies in the field of prevention, including cost-effectiveness analyses, outcomes research or preference studies. As preventive health interventions are complex, development in methods may also be expected to facilitate the effectiveness and cost-effectiveness evaluation of these interventions. The COVID-19 pandemic has further highlighted the importance of research about preventive and public health measures to better equip countries for future pandemics.

7. Evaluating and Assessing Medications for Rare Conditions

We are currently in an era of unprecedented growth in the development and utilization of so-called “orphan” drugs for the treatment of rare diseases.²⁸ Orphan drugs, designed for diagnosing, preventing, or treating rare diseases, now play a pivotal role in healthcare. Many rare cancers, including pediatric cancers, fall into this category, and oncology indications now make up more than a third of these drugs. It is projected that by 2022, orphan drugs will constitute over 20% of pharmaceutical spending in developed nations.²⁹ The influx of new regulatory submissions for orphan indications has reached record levels, with the FDA granting orphan designation to 54% of drugs

approved in 2022.³⁰ This growth brings both challenges and opportunities for US healthcare policymakers. They must establish a clear framework for evaluating and pricing orphan drugs, drawing insights from international experiences and addressing unique ethical and contextual considerations.

This expansion has been facilitated by legislative and regulatory incentives in the United States and other countries. However, as orphan drug utilization increases, access to these treatments becomes more challenging. The cost-effectiveness of orphan drugs is often uncertain, and payers must strike a balance between accommodating crucial innovations and managing rising costs.³¹

The orphan drug landscape is evolving rapidly, offering hope for patients but also causing concern about healthcare budgets.³² These drugs are no longer a minority in the realm of drug approvals. Furthermore, the prevailing notion that higher “orphan drug prices” were necessary to ensure reasonable profits for innovators is also under scrutiny. Additionally, determining what constitutes a “reasonable” innovator profit is a complex issue, further complicated by government subsidies for orphan drug development, which offset significant clinical development costs, provide tax incentives, and extend patent protections. In summary, the surge in orphan drugs brings immense promise and uncertainty. Policymakers must navigate these complexities and adapt traditional methods of health technology assessment and economic evaluation to accommodate the unique landscape of rare diseases.

8. Outcomes-Based Contracting

Outcomes-based contracting (also referred to as performance-based risk-sharing arrangements, value-based contracts, among other terms) may very well prove to be the future of pharmaceutical pricing and reimbursement. Such arrangements have gained in relevance with the increasing number of high-cost therapies, especially in areas with unmet medical needs or where conventional cost-effectiveness evaluations are challenging. Most current methods of outcomes-based contracting link payment for a drug to its actual performance in real-world settings and the clinical outcomes achieved. In other words, a “reward for good work” or “punishment for poor work”: respectively, more money or less money for the manufacturer and, conversely, less money or more money for the payer. Further, by considering (only) real-world data, there is an inherent time lag that may easily amount to 2–3 years to achieve sufficiently stable and robust clinical effectiveness data.

The advantages are important nonetheless: financial benefits for payers, better access to treatment for patients, and incentives to innovate for manufacturers. On the other hand, there are some challenges and burdens: defining and measuring outcomes of interest, which currently is limited mainly to outcomes of benefit; data acquisition and analysis; time horizon; management; dispute resolution; and the potential for overly complex agreements.^{33,34}

Most outcomes-based contracting models developed to date come with notable limitations. There is a lack of independence as the contracting about price and the associated price elasticity in the payback scenarios is negotiated between two parties with vested financial interests.^{35,36} These interests may not align in terms of outcomes and converge in terms of price. A focus on clinical benefit prevails and the avoidance of harm is seldom considered. The impact of patients’ medication behavior and its impact on clinical outcomes is not considered. Parties may differ, in concept and in operationalization, as to what constitutes benefit and treatment success and what price points and ranges are defensible and affordable for all payers, patients included – foregoing transparency in the process. Alkhatib and colleagues developed the Six Delta platform for outcomes-based contracting for pharmaceuticals, “an independent platform that supports joint and equitable price negotiation” that “should assure better coverage and patient access, stimulate payers and their providers under contract with value incentives to achieve more beneficial health outcomes and reduce the cost wastage that comes from mismatching treatments with patients’, while also lowering “patients’ share of treatment cost”. Based on prediction and simulation modeling, the platform integrates six pricing dimensions: pricing-based cost-effectiveness and cost-utility analysis; pricing based on willingness-to-pay; reference-based pricing; safety-based pricing; pricing based on risk of efficacy failure; and adherence-based pricing.³⁵ This model has attracted quite some interest from the payer community.

9. What Makes a High Quality HEOR Paper – An Editor’s Perspective

Manuscripts that report on HEOR may contain jargon that is difficult for the average reader of a clinical journal to understand. Another potential problem is inadequate discussion as to what the results may mean to the clinical stakeholders – health care providers, their patients, and the patients’ families. Best practices would ensure the inclusion of contributors who can opine on these matters, including at the start of the project, even though the technical execution of the economic analyses would be beyond their usual scope of work. Clinical context may not be as relevant for highly specialized journals that principally have a non-clinical audience, but a plain language summary would remain helpful.

Journal editors seek and welcome submissions that are of sufficient interest to generate citations. Where the research originates can play a significant role in how seriously it is considered. For example, a high value is given to work generated through a rigorous peer-directed funding process by governmental/quasi-governmental agencies or independent NGOs/NFPs whose mission is to do this kind of work. The resultant projects are usually well-thought through and can be highly influential. An example is that of the Institute for Clinical and Economic Review,³⁷ with its transparent processes for solicitation of public comments.

Industry-directed HEOR is also a common source of submission of papers to a wide array of medical journals, including those journals where HEOR manuscripts comprise a small proportion of their content but where the topic may be highly relevant to clinical decision-making. This may include reports from commercial entities wishing to raise awareness about a disease state and/or intervention. Good Publication Practice guidelines for company-sponsored biomedical research offer guidance regarding the inclusion of HEOR within a commercial entity’s Standard Operation Procedures for publication management,³⁸ however in many instances such work is outsourced to external vendors who have experience with analysing large claim databases. As mentioned earlier, clinical input from clinician-researchers in the specialty of the topic under discussion is highly recommended at all stages of an HEOR project. The lack of a central registry for HEOR work prior to the commencement of the project hampers transparency, and it can be assumed that some analyses are undertaken and never published, as had been the situation in the past with negative or failed clinical trials prior to the introduction of requirements for registration in databases such as ClinicalTrials.gov or EudraCT.³⁹

Some HEOR research does not require the collection of original data but can be accomplished with existing information, including extensive datasets that can be accessed at little or no cost, such as the National Health and Nutrition Examination Survey⁴⁰ and the Medical Information Mart for Intensive Care.⁴¹ This can lead to academic exercises or “data mining” and result in manuscripts that contribute little in the way to answering questions relevant to clinical decision-making. This is akin to the creation of meta-analyses that are developed in the absence of clinical wisdom and that often provide little in the way of clinical context as to their purpose.⁴² An indicator that this may be the case is a submission describing data from another country whose health care system is very much different from that of the authors, and/or in an area of specialty that differs from the authors expertise, and/or appears to be the work of an individual or group who have grasped on to a formulaic strategy of publishing similar papers (akin to what editors have encountered in terms of “serial letter writers”).

Lastly, securing useful and actionable peer review can be challenging as a number of different skill sets are required to evaluate HEOR papers. Not all peer reviewers can necessarily address all the components of the paper. It may be useful to expand the pool of potential reviewers by setting expectations in advance such that a non-statistician would not be expected to review highly technical methodology (other than comment if the language can be made clearer), and a statistical reviewer would not be expected to comment on clinical context.

Concluding Remarks

We hope you have found this helpful and that you have enjoyed reading about what we believe will trend in health economics in 2024. If you are interested in finding out more about the EiCs that authored this article, their bios are available at the end of this article.

We encourage our authors and audience to contact us with submission inquiries, ideas for articles and special issues, and queries about supporting the journal as an Editorial Board member or peer reviewer. Details on how to contact us are available in [Table 1](#).

Table 1 Provides Contact Details for the Editors That Manage the Journals

Journal	Link to Aims and Scope	Executive Editor and Email Address
Expert Review of Pharmacoeconomics and Outcome Research	Aims and scopes	Rayya Hajjar
ClinicoEconomics and Outcomes Research	Aims and scopes	Alexandra Carrick
CMRO	Aims and scopes	Mithi Ahmed
Journal of Medical Economics	Aims and scopes	Mike Gregg

Biography

Ivo Abraham is a Professor of Pharmacy, Medicine, and Clinical Translational Sciences at the University of Arizona and is also EIC of the Journal of Medical Economics. His knowledge and experience of the HEOR field is extensive, and he has particular interests in clinical outcomes and effectiveness research as well as value appraisal from molecules to models of care. He has (co-)authored 500+ articles and 80+ chapters and has (co-)edited 50+ books, monographs, and journal issues. His group was among the first to recognize the economic importance of biosimilars in terms of health equity and access to care, and he writes a column for the AJMC Center for Biosimilars. He has been affiliated with universities in the US, Europe, and Asia.

Mickael Hiligsmann is Associate Professor in Health Economics and Health Technology Assessment at Maastricht University, the Netherlands, and is also EIC of Expert Review of Pharmacoeconomics & Outcomes Research. He holds two PhD degrees from Maastricht University and the University of Liège, a master in Public Health and a master in Economics. His research expertise and interest includes health technology evaluation (such as cost-effectiveness analyses, decision-analytic modelling, quality of life), the elicitation of preferences in health care, and medication adherence and patient involvement. He has been co-promotor of about 25 PhD students and is senior lecturer in HTA and health economic courses. He is currently author of about 275 peer-reviewed articles (with about 50 as first-author). He is the coordinator of the Maastricht Health Economics and Technology Assessment center.

Leslie Citrome is clinical professor of psychiatry and behavioral sciences at New York Medical College in Valhalla, New York, and Immediate Past-President of the American Society of Clinical Psychopharmacology, and a member of the Board of Directors of the World Association of Medical Editors. Dr. Citrome is a frequent lecturer on the quantitative assessment of clinical trial results, including the metrics of number needed to treat and number needed to harm. His main interests include schizophrenia, bipolar disorder, and major depressive disorder. He is author or coauthor of over 600 research reports, reviews, and chapters in the scientific literature. In 2019, he received the New York Medical College Faculty Author Award for “First Authorship Who Published in the Most Open Access Journals (Web of Science)”, He has a private practice in psychiatry in Pomona, New York and is a volunteer consultant to the Assertive Community Treatment team/Mental Health Association of Rockland County.

Kenneth Lee is Professor of Health Economics at Taylor’s University Malaysia. Previously he had held senior academic positions in Hong Kong and the UK. He has been EIC of JME since 2006. His area of research interest is healthcare evaluation at both micro and macro levels. Over the years, he has worked on research projects in collaboration with different clinical specialities including cardiology, dermatology, gastroenterology, infectious diseases, oncology, public health and psychiatry. He served in a number of important positions in the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) including member of the Board of Directors and Co-editor in Chief of Value in Health Regional Issue. He has published extensively in international peer-reviewed journals with a few high-impact articles that had led to changes of international clinical management guidelines.

Giorgio Lorenzo Colombo is an adjunct Professor at the University of Pavia – School of Pharmacy and brings to his work a notable combination of academic theory and analytical practice. His experience in independent consultancy has seen him perform technological assessments and economic evaluations for pharmaceutical companies and Italian governmental authorities alike, while maintaining a highly active research portfolio. Much of his recent research has focused on the economic evaluations of Drugs and Medical Device, showcasing his ability to bring together theoretical

models with their social and business applications. Since graduating from the Catholic University of Milan, Professor Colombo has authored or co-authored about 220 scientific papers and three books in the fields of health economics and pharmacoeconomics research, as well as sitting on the editorial boards of several scientific peer review journals.

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