ISPE position statement on strengthening evidence-based medicine in population health

Scientific advances in prevention, diagnosis, and treatment have transformed population health worldwide, contributing to a global increase in life expectancy of more than 40 years over the past century (Roser et al, 2023). These gains have been achieved through a range of medical innovations, including vaccination programs, screening initiatives, modern imaging, biomarkers, targeted therapies, minimally invasive surgical techniques and ensuring access to health services (Shattock et al 2024, GBD 2021).

Despite this progress, health systems globally face increasing challenges to maintain population health and ensure equitable access within resource constraints. The increasing gap between needs and resources is driven by both demand side forces (aging populations and patient expectations) and supply side forces (introduction of new pharmaceuticals and medical devices combined with the rapid development of digital health technologies) (Barker 2011). To maintain population health and ensure equitable access, it is essential to identify and reduce low-value care and improve health-care quality by implementation of evidence-based medicine (EBM).

This position statement reaffirms the central role of scientific evidence in clinical practice and healthcare decision-making. It emphasizes the urgent need to protect and strengthen evidence-based systems in an increasingly complex and contested information environment (Oxman et al 2025). This statement builds on the International Society for Pharmacoepidemiology's (ISPE) longstanding mission to generate, critically appraise, and translate high-quality evidence to inform clinical, payer, regulatory, and public health decisions.

The Foundational Role of Scientific Evidence in Health Practice and Policy

Evidence-based medicine integrates the best available research evidence with clinical expertise and patient values to support informed decision-making (Sackett et al, 1996). In public health, evidence-informed decision-making requires systematic identification, appraisal, and application of reliable evidence to guide policies and programs.

The World Health Organization's evidence-informed decision-making framework emphasizes multidisciplinary assessment, transparent translation of research into policy, and meaningful integration of epidemiological data, analytic methods, and patient perspectives. Evidence-based medicine is essential for designing effective interventions, allocating resources fairly, and advancing sustainable development goals (World Health Organization 2021, Wieringa et al 2023). These principles indicate that health-care interventions should be scientifically rigorous, socially responsive and contextually appropriate.

The Value of Research for Improving Population Health Outcomes

Evidence-informed policymaking emphasizes that policy decisions should be informed by the best available evidence on effectiveness, equity, feasibility of implementation, affordability, sustainability, and acceptability to stakeholders (WHO 2021). Policies informed by research evidence will be more effective, need less resources, have more legitimacy, and ensure transparency and accountability in decision-making (Haby et al 2025, WHO 2021).

An evidence-based public health framework emphasizes:

- Community assessment and engagement
- Quantification of public health problems using surveillance and population data
- Identification and prioritization of effective interventions
- Rigorous evaluation of programs and policies
- Broad dissemination of evidence to stakeholders

Together, these components support equitable and informed decision-making, especially in resource-constrained settings (Jacobs JA et al 2012, Brownson et al 2026)

Innovative and Advanced Therapies: Evidence Uncertainty and System-Level Impact

Recent advances in oncology and in the treatment of rare diseases have accelerated the development and approval of innovative therapies, often based on limited clinical evidence-such as small, single-arm trials lacking randomization, adequate comparator groups or long-term follow-up (Hatswell et al, 2023, Iglesias-Lopez et al 2021). While expediated pathways may be justified in areas of high unmet need, they introduce significant uncertainty regarding true clinical benefit, long-term safety, and population-level effectiveness.

These challenges are amplified when such technologies are rapidly adopted into publicly funded health systems, often at very high prices and with substantial budgetary impact. For low- and middle-income countries and countries with universal health systems, independent and locally generated real-world evidence (RWE) becomes essential to assess performance, value, and equity considerations. (Verkerk & Voest 2024). Technologies developed elsewhere may perform differently in populations with distinct epidemiology, genetics, health system structures, and care pathways.

Ensuring equitable and sustainable access requires scrutiny of both the evidentiary basis and the real-world performance of advanced therapies, informed by robust pharmacoepidemiological methods.

Integrating Research into Clinical and Public Health Decisions

High-quality evidence synthesis-including systematic reviews and meta-analyses - remains the gold standard for translating complex bodies of evidence into actionable guidance. Cochrane methodologies and robust health policies such as those provided by ISPE aim to minimize bias and deliver reliable, decision ready insights.

In public health policy, Evidence-to-decision frameworks enhance transparency and legitimacy by explicitly considering benefits and harms, equity, feasibility, resource implications and governance processes such as conflict-of-interest management (Alonso-Coello et al., 2016). Building capacity in evidence appraisal and use is essential: modular training in EBM and public health policy equips clinicians, policy makers, patients and community stakeholders with the skills needed to interpret evidence, prioritize options and evaluate impact.

Protecting and Strengthening Evidence-Based Approaches Against Misinformation

The spread of health-related misinformation poses a profound threat to evidence-based practice. Digital platforms can rapidly disseminate false or misleading health information, eroding trust in institutions, reducing uptake of effective interventions and undermining public health initiatives (Rodriguez et al 2024, Gram et al 2025). Research shows that emotional content, political identity, and repeated exposure increase the likelihood of misinformation being accepted and shared (Sallam 2025).

The World Health Organization characterizes this challenge as an "infodemic" -an overwhelming volume of information that makes it difficult to identify trustworthy sources (Ishizumi et al 2024). Safeguarding evidence-based medicine and public health requires strategies to counter misinformation, protect scientific processes from political and commercial interference trust (Abdekhoda and Dehnad, 2025), and promote scientific and health literacy, health literacy across all sectors (Wojtowicz, 2020).

In addition to traditional forms of misinformation, emerging digital technologies are reshaping the evidence ecosystem. The rapid expansion of artificial intelligence (AI), including large language models (LLMs), presents both significant opportunities and emerging risks for evidence generation and dissemination. AI tools can enhance literature synthesis, signal detection, data analysis, and knowledge translation at unprecedented scale. However, when used without appropriate methodological oversight and domain expertise, these systems may generate inaccurate, non-reproducible, or fabricated content ("hallucinations"), potentially amplifying misinformation or creating a false sense of evidentiary certainty. Safeguarding scientific integrity in the era of AI requires transparency, validation standards, human oversight, and clear accountability in the use of AI-supported research outputs.

The Contribution of Real-World Evidence and Pharmacoepidemiology

High-quality, independent real-world evidence (RWE)-grounded in robust local data and rigorous epidemiologic methods-is indispensable for evaluating whether innovative and advanced therapies deliver meaningful benefit in routine practice. Real-world studies provide insight into patterns of medication use, effectiveness, safety and health outcomes beyond the controlled clinical trial environment.

Health technology assessment and health service evaluation should therefore be core components of evidence-based practice. These require transparent protocols, appropriate comparators, robust methods, including those for causal inference, and meaningful outcome measures. When systematically implemented, RWE generation supports accountability, informs adaptive pricing and coverage decisions, and strengthens public trust.

Randomized controlled trials remain a key part of EBM, however, many health systems now employ entry agreements and performance-based arrangements to balance early access with the need for post-launch evidence highlighting the need for robust pharmacoepidemiological methods. RWE derived from real-world data (RWD), including electronic health records, claims, registries, and other sources, provides essential insights across the product life cycle including post-marketing safety surveillance, comparative effectiveness, and label expansion providing important insights into health care utilization to inform decision-making. Pharmacoepidemiology leverages RWD for health-care decision-making using proven methodological approaches such as marginal structural models, time-related bias mitigation, self-controlled designs, propensity score techniques, negative control methods, new user approaches, use of external controls and pragmatic randomized clinical trials providing RWE to support EBM. Together, these methods support rigorous evaluation of benefits and harms in diverse populations and over longer follow-up periods than is typically feasible in traditional trials.

The International Society of Pharmacoepidemiology makes a significant contribution to evidence-based decision-making via:

- **Methodological leadership** in advanced pharmacoepidemiologic designs
- **Lifecycle evidence generation** for medicines and medical devices across development, regulatory review, reimbursement, and post-marketing phases.
- **Standards and guidance** for research transparency, fit-for-purpose, external controls and quality assurance.
- **Education and capacity building** to equip the global workforce to produce credible RWE, including use of novel data sources such as patient-generated data, natural language processing and AI.

Key Recommendations to Strengthen Evidence-Based Decision-Making

To advance population health in an era of increasing complexity and information challenges, ISPE calls for:

1. **A renewed commitment to evidence-based medicine and public health** as foundational principles across health systems.
2. **Sustained investment** in transparent, high-quality research, rigorous evidence synthesis and capacity building.
3. **Institutional safeguards** to protect scientific processes from political and commercial influence.
4. **Comprehensive strategies to counter misinformation** through regulatory oversight, digital platform accountability and public education.
5. **Promotion of scientifically informed decision-making** across clinical, government, educational, and community-based sectors.
6. **Expanded education and training** in EBM and public health at undergraduate, postgraduate and continuing professional levels.
7. **Strengthened cross-sector collaboration** among scientific organizations, policymakers, journalists, patient groups and community leaders to reinforce public trust in evidence-based practice.

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