

# 1 International Society for Pharmacoepidemiology in 2 Action: Advancing Public Health Through Evidence- 3 based Advocacy

4  
5 **Authors: Sarri G, Diaz-Decaro J, Burcu M, Soriano Gabarró M, Gini R, Bahri P, Ritchey**  
6 **ME**

## 7 **Author Affiliations:**

- 8 • Grammati Sarri, PhD – Vice President Innovative Statistics, Evidence, Value, Access and  
9 Health Policy, Cytel, Inc.
- 10 • John Diaz-Decaro, PhD – Senior Director and Lead Epidemiologist, ModernaTX, Inc
- 11 • Mehmet Burcu, PhD, MS—Senior Director, Epidemiology, Merck & Co., Inc., Rahway,  
12 NJ, US
- 13 • Montse Soriano Gabarró, MD, MSc, FISPE, Independent researcher, Potsdam, Germany
- 14 • Rosa Gini, PhD – Head of Pharmacoepidemiology Unit, ARS Toscana, Italy
- 15 • Priya Bahri, PhD, Senior Lead, Pharmacovigilance, European Medicines Agency (EMA),  
16 Amsterdam. Netherlands
- 17 • Mary E Ritchey, (1) Principal and Owner, Med Tech Epi LLC; (2) Associate Research  
18 Professor, Center for Pharmacoepidemiology and Treatment Science, Institute for Health,  
19 Health Care Policy, and Aging Research & Center for Health Outcomes, Policy, and  
20 Economics Rutgers University, New Brunswick, NJ, US

21 Correspondance: Grammati Sarri, [grammati.sarri@cytel.com](mailto:grammati.sarri@cytel.com)

22 Keywords: advocacy, ISPE, pharmacoepidemiology, evidence policy-making, public health

## 23 **Key points:**

24 Public health is facing increasingly complex challenges, including emerging diseases, widening  
25 health disparities, environmental risks, and widespread misinformation—intensified by financial  
26 and geopolitical pressures. In this context, translating scientific evidence into effective policy  
27 requires purposeful, sustained advocacy to bridge knowledge and action. Advocacy is central to  
28 ISPE’s mission, supported by a bottom-up, transparent governance model that engages members,  
29 committees, and the Board to shape positions and collaborations with key global partners. In  
30 2025, ISPE advanced multiple long- and short-term initiatives and continues to embed advocacy  
31 within its strategic plan to strengthen public health impact, capacity building, and global  
32 evidence-to-policy translation.

### 33 Plain language summary:

34 Public health is dealing with many challenges today, such as new diseases, growing health  
35 inequalities, environmental risks, and extensive misinformation. To protect people’s health,  
36 scientific evidence needs to be turned into public policies, and this requires active and ongoing  
37 advocacy. ISPE plays an important role in this work by helping ensure that research on how  
38 medicines are used and how safe they are can guide decisions made by regulators, clinicians, and  
39 public health leaders. Through a transparent, member-driven process and strong partnerships,  
40 ISPE leads advocacy efforts worldwide and continues to build capacity so that evidence can  
41 better support public health.

42

### 43 Introduction

44 Public health faces increasingly complex challenges including emerging infectious diseases,  
45 rising non-communicable and mental health burdens, widening health disparities and  
46 environmental risks, emerging technologies and public health ethics, healthcare costs and  
47 underinvestment in health systems (1, 2). These challenges are amplified by financial pressures,  
48 geopolitical instabilities (3) and the spread of medical and public health misinformation and  
49 disinformation (4). In this context, translating scientific knowledge and advancements into  
50 effective, evidence-informed policy is essential for strengthening public health and improving  
51 patient outcomes (5). That translation does not happen automatically; it requires deliberate,  
52 sustained advocacy—bridging evidence and action and ensuring equitable access and improved  
53 population health and wellbeing.

54 Advocacy is central to the International Society for Pharmacoepidemiology’s (ISPE) mission.  
55 ISPE aims to advance the public health by providing a global forum for scientific innovation,  
56 engagement and collaboration through leadership in the field of pharmacoepidemiology (6).  
57 Through both long-term strategic initiatives and activities (7), ISPE promotes the use of  
58 pharmacoepidemiologic methods and real-world evidence (RWE) in shaping regulatory science  
59 and public-health decision-making (8).

### 60 How is Public Health Advocacy Through Pharmacoepidemiology

#### 61 Accomplished by ISPE?

62 ISPE advocates for translating pharmacoepidemiologic evidence into meaningful public health  
63 action through a structured, transparent, member-driven process. Advocacy activities span the  
64 full continuum from early horizon scanning and identifying emerging issues relevant to public  
65 health, to formal engagement in open calls for comment to regulatory and policy documents, and  
66 strategic collaboration with other aligned organizations such as European Network of Centres for  
67 Pharmacoepidemiology and Pharmacovigilance (ENCePP), the Council for International  
68 Organizations of Medical Sciences (CIOMS), the International Network for Epidemiology in

69 Policy (INEP), and other clinical research and professional societies. These efforts ensure that  
70 pharmacoepidemiologic evidence is timely, relevant and positioned to inform decision-making  
71 across regulatory, clinical, and public health contexts.

72 Central to this approach is ISPE’s bottom-up governance model. Advocacy topics originate from  
73 the Society’s diverse global membership and are refined through structured review by the Public  
74 Policy Committee (PPC) and the Strategic Planning Committee (SPC). The Board holds the final  
75 responsibility for initiation of advocacy efforts as well as endorsement and approval of official  
76 Society positions. This inclusive model promotes transparency, contribution, representative  
77 expertise and perspectives, and trust within the Society and among external stakeholders, while  
78 cultivating leadership and advocacy skills among members from diverse professional,  
79 geographic, and cultural backgrounds. Advocacy for ISPE is a collective commitment shaped by  
80 a structured process with pre-defined steps and activities.

81 In 2025, ISPE led 5 longer-term and 8 short-term strategic efforts, in addition to ongoing  
82 collaborations with 10 strategic partner organizations. The sections below offer examples of  
83 ISPEs advocacy efforts:

#### 84 1. Identification of Long-standing and Emerging Issues for Advocacy

85 Early identification of health and research topics of public health interest and of the need for  
86 leading advocacy efforts, either in short- or long-term action plans, is part of the key strategic  
87 initiatives for ISPE. It occurs in structured and systematic processes through two mechanisms—

88 (1) a biannual evaluation and identification of emerging topics and advocacy issues (9): ISPE’s  
89 PPC conducts biannual horizon scanning and summarizes proposed emerging and advocacy  
90 issues as submitted by ISPE members for prioritization discussions at ISPE’s Strategic Planning  
91 Committee. An *emerging issue* is defined as a topic of relevance to pharmacoepidemiology that  
92 is new to ISPE or which requires significant further development and guidance in terms of  
93 definition or methodological practices for appropriate application. Advocacy issues are defined  
94 as those which require ISPE’s consideration for advocacy aligned with its mission and goals and  
95 are considered actionable or otherwise can be influenced by ISPE advocacy efforts (9).

96 (2) an ongoing opportunity for any member of ISPE to propose a potential strategic effort  
97 throughout the year (8). Long-term strategic efforts are reviewed by the SPC with a  
98 recommendation provided to the Executive Committee and short-term strategic efforts are  
99 reviewed by the Executive Committee directly.

100 Advocacy issues can be long-standing or emerging. In both processes, the final decision on  
101 prioritization and selection of topics for actionable planning as part of ISPE’s goals is made by  
102 the Executive Committee or Board based on considerations of public health importance,  
103 potential for impact, and anticipated resources needed. As part of this process, topics are  
104 assigned to one of three priority levels: 1) new strategic efforts supported and monitored by the  
105 Board; 2) non-strategic activities that may be supported by individual ISPE Communities (e.g.,

106 regional or special interest groups); or 3) topics for which ISPE engagement is not  
107 recommended.

## 108 2. Public Commentary

109 ISPE is committed to fostering open and informed debate of matters of scientific and  
110 professional importance and to providing the public with the Society's collective expertise on  
111 issues of public health relevance, particularly in circumstances of urgency or broad societal  
112 impact. ISPE may officially respond to public consultations of regulatory or relevant  
113 international scientific documents by convening ISPE working groups with expertise in a related  
114 field to lead the development of public statements on behalf of ISPE. The endorsement process  
115 of these statements is coordinated by the PPC through a stepwise process of full membership  
116 review, and formal endorsement via the Board (Table 1 provides examples of ISPE 2025  
117 activity).

## 118 3. Thought Leadership Publications and Responses to Emerging Challenges

119 Shaping good practice methods standards by driving innovation and strengthening  
120 methodological capacity remains one of ISPE's core functions. As the field evolves, ISPE plays a  
121 critical role in identifying methodological gaps that may affect the quality, interpretability, and  
122 credibility of evidence used to inform public health and regulatory decision-making.

123 For example, an emerging challenge identified by the ISPE Databases SIG was increasing  
124 consistent reporting on data diversity which is required to enhance study interpretability and  
125 support replicability. In response, the *DIVERSE* framework (10), derived from a systematic  
126 scoping review was developed and is now being operationalized into a tool that will be used for  
127 that purpose. Likewise, the Reporting Recommendations Intended for Pharmaceutical Risk  
128 Minimization Evaluation Studies: Standards for Reporting of Implementation Studies Extension  
129 (RIMES-SE) was developed within ISPE (11) and is among the checklists now encouraged for  
130 use by EU regulatory guidance to strengthen the quality, evidence and interpretability of study  
131 results for decisions on risk minimization interventions (12).

132 Thought leadership publications or other position statements are developed by working groups,  
133 reviewed by the ISPE membership, and endorsed by the Board before submission for  
134 publication.

## 135 4. Strategic Partnerships and Other Collaborations

136 ISPE actively builds strategic collaborations across sectors to amplify the impact of  
137 pharmacoepidemiology science on public health policy and practice. To support this goal, the  
138 ISPE Board selects members with leadership experience to act as strategic liaisons with external  
139 organizations to identify opportunities for collaboration, including advocacy efforts.

140 Examples of these collaborations are position statements developed in collaboration with the  
141 INEP (13) emphasizing the importance of conflict-of-interest disclosure in epidemiological

142 research to safeguard scientific integrity, as well as leadership of an INEP article establishing the  
143 concept of epidemiological literacy and calling for its strengthening across policy and public  
144 health communities (14).

145 Other collaborations include joint advocacy to advance understanding of pharmacoepidemiology  
146 and other outcomes research, such as the ongoing Real World Evidence Transparency Initiative  
147 involving ISPE, the International Society for Pharmacoeconomics and Outcomes Research  
148 (ISPOR), the Duke-Margolis Center for Health Policy, and the National Pharmaceutical Council  
149 which has led to the development of a real-world evidence registry for studies before their start  
150 to increase reproducibility (15). Another effort is the HARmonized Protocol template to Enhance  
151 Reproducibility (HARPER) which was recognized in the International Council for  
152 Harmonization (ICH) guideline (16) and is now recommended by regulators (17) and payers  
153 (18).

## 154 5. Long-term Vision

155 Advancing advocacy in pharmacoepidemiology over the long term is central to ISPE’s mission  
156 to improve public health through scientific exchange, methodological innovation, education,  
157 leadership, and policy engagement. The ISPE Strategic Plan (2024–2029) explicitly positions  
158 advocacy as a core mechanism for supporting and translating pharmacoepidemiologic evidence  
159 into public health policy and clinical practice across diverse global contexts (7).

160 To operationalize this vision, ISPE is committed to completing at least one advocacy-aligned  
161 activity annually in at least of 90% of its ongoing external partnerships and embedding advocacy  
162 within sustained scientific collaboration. Importantly, ISPE’s long-term advocacy vision is also  
163 tightly linked to capacity building and empowerment.

164 By increasing opportunities for engagement, leadership development, and cross-sector  
165 collaboration, particularly for early- and mid-career professionals and members from  
166 underrepresented regions, ISPE aims to cultivate a globally distributed community capable of  
167 contributing to and leading evidence-informed policy discussions. Through this approach,  
168 advocacy serves not only as a vehicle for influencing policy, but also as a means of strengthening  
169 the field of pharmacoepidemiology itself and ensuring its relevance, credibility, and equity in  
170 shaping public health decisions worldwide.

### 171 **Table 1. Examples of ISPE Public Commentary to Draft Regulatory Guidance in** 172 **2025**

During 2025, ISPE responded to the following draft US Food and Drug Administration (FDA) guidance documents. A summary of ISPE’s response is provided below highlighting opportunities for advocacy in public health while emphasizing methodological topics in each response.
--

<b>1. Considerations for the Use of Artificial Intelligence (AI) to Support Regulatory Decision-making for Drug and Biological Products (19)</b>
--

The Society’s feedback emphasized several key recommendations to strengthen the draft guidance including aligning AI/machine-learning terminology and encouraging practical application of the framework in real-world settings, especially in pharmacovigilance and drug safety. To ground the risk-based framework in practical application, we proposed real-world use cases—such as AI-driven pharmacovigilance systems that analyze electronic health records to detect adverse drug reactions—highlighting how the framework could be applied in post-market safety surveillance. We also stressed the importance of lifecycle management, especially for self-evolving systems, and recommended the establishment of a centralized, public-facing repository of validated AI models, like the EU AI Act’s registry for high-risk models, to enhance transparency and reusability. Lastly, we called for greater attention to model provenance and governance, encouraging the FDA to consider frameworks from organizations like the National Telecommunications and Information Administration and to set clear expectations around documentation, versioning, and traceability that are proportional to the model’s risk and regulatory impact.

## **2. Considerations for the Study of Sex Differences in the Clinical Evaluation of Medicinal Products (20)**

Several key points were raised in ISPE’s response, of which the distinction between sex and gender, the matter of lack of representation of women in clinical trials, and the fact that limited or lack of data on sex differences should not be translated as the absence of sex differences. Further, emphasis was placed on a historic inattention paid to female data in specific diseases (e.g., cardiovascular and Parkinson’s disease) due to a lack of understanding of biological differences and the importance of applying specific statistical concepts to fully support hypothesis-driven analyses powered to conduct sex-based analysis in clinical trials. In addition, ISPE highlighted issues, such as the underrepresentation of women in the training, testing, and validation of AI models, and the types of biases—whether intentional or unintentional—that can lead to inaccuracies and further threaten to address the needs of marginalized trial participants and to adversely affect downstream users of clinical data.

## **3. Considerations for the Collection and Submission of Patient Preference Information (PPI) Across the Total Product Lifecycle (21)**

Comments from ISPE members included requests for additional clarity regarding the Agency’s recommendations to ensure that sponsors’ investment in this research is valuable for decision-making. ISPE’s comments focused on areas in which sponsors may seek clarification including:

- Context beyond benefit risk assessment in which PPI can be useful to support sponsors’ evidence planning
- Guidance or examples to guide sponsors in the design and execution of formative qualitative preference research
- Context and applications along with the total product lifecycle in which preferences of cohorts other than (or in addition to) patients would be considered by FDA
- Additional methodological guidance, including for example, the types of preference heterogeneity analysis and the selection and interpretation of assessments of response consistency

174 **Disclaimer**

175 The views in this article are the authors' personal views and must not be taken as the views of  
176 their respective employers.

177 **Acknowledgements**

178 We acknowledge Christine Poulos for leading ISPE response to the FDA draft guidance on PPI  
179 across the total product lifecycle.

180 **Conflicts of Interest**

181 G.S. is employed and holds Cytel shares and non-paid Board positions at ISPE and to a  
182 European- funded AI trial in breast cancer (<https://cinderellaproject.eu/>). JDD is an employee at  
183 Moderna and hold stock options and RSUs. MB is an employee of Merck Sharp & Dohme LLC,  
184 a subsidiary of Merck & Co., Inc. and owns stock in Merck & Co., Inc. MSG is a former  
185 employee of Bayer AG and GSK and holds shares in GSK. RG is employed by ARS Toscana, a  
186 publicly owned research center that conducts studies funded by private and public institutions  
187 and compliant with the ENCePP Code of Conduct. MB and MSG are editorial board members of  
188 Pharmacoepidemiology and Drug Safety and co-authors of this article. They were excluded from  
189 editorial decision-making related to the acceptance of this article for publication in the journal.

190 **References**

- 191 1. Lucero-Prisno DE, 3rd, Shomuyiwa DO, Kouwenhoven MBN, Dorji T, Adebisi YA, Odey  
192 GO, et al. Top 10 Public Health Challenges for 2024: Charting a New Direction for Global Health  
193 Security. Public Health Chall. 2025;4(1):e70022.
- 194 2. Reid MJA, Katz IT. Redefining global health in the 21st century: Towards a more equitable  
195 global health agenda. Global Public Health. 2025;20(1):2464060.
- 196 3. Lastuka A, Breshock MR, Hay SI, Taylor KV, Lim SS, Murray CJL, et al. Global, regional, and  
197 national health-care inefficiency and associated factors in 201 countries, 1995&#x2013;2022: a  
198 stochastic frontier meta-analysis for the Global Burden of Disease Study 2023. The Lancet  
199 Global Health. 2025;13(8):e1349-e57.
- 200 4. Khullar D. Social Media and Medical Misinformation: Confronting New Variants of an Old  
201 Problem. JAMA. 2022;328(14):1393-4.
- 202 5. Suazo-Galdames IV SM, Chaple-Gil AM,. Scientific evidence and public policy: a  
203 systematic review of barriers and enablers for evidence-informed decision-making. Frontiers in  
204 Communication. 2025;10.
- 205 6. International Society for Pharmacoepidemiology. About ISPE [Available from:  
206 <https://www.pharmacoepi.org/about-ispe/overview/>].
- 207 7. International Society for Pharmacoepidemiology. Strategic Plan 2024–2029 [Available  
208 from: Scientific evidence and public policy: a systematic review of barriers and enablers for  
209 evidence-informed decision-making.
- 210 8. International Society for Pharmacoepidemiology. Policy Manual 2025 [Available from:  
211 <https://www.pharmacoepi.org/pub/?id=FF2E0B27-E5F2-877C-B787-3E95D07D0611>].

- 212 9. International Society for Pharmacoepidemiology. Strategic Initiatives [Available from:  
213 <https://www.pharmacoepi.org/strategic-initiatives/ispe-emerging-advocacy-issue-checklists/>.  
214 10. Gini R, Pajouheshnia R, Gardarsdottir H, Bennett D, Li L, Gulea C, et al. Describing  
215 diversity of real world data sources in pharmacoepidemiologic studies: The DIVERSE scoping  
216 review. *Pharmacoepidemiol Drug Saf.* 2024;33(5):e5787.
- 217 11. Smith MY, Morrato EH, Mora N, Nguyen V, Pinnock H, Winterstein AG. The Reporting  
218 Recommendations Intended for Pharmaceutical Risk Minimization Evaluation Studies: Standards  
219 for Reporting of Implementation Studies Extension (RIMES-SE). *Drug Safety.* 2024;47(7):655-71.
- 220 12. European Medicines Agency. Guideline on good pharmacovigilance practices (GVP);  
221 Module XVI – Risk minimisation measures (Rev 3) **and its Addendum II** 2024 [Available from:  
222 [https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/guideline-good-](https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/guideline-good-pharmacovigilance-practices-gvp-module-xvi-risk-minimisation-measures-rev-3_en.pdf)  
223 [pharmacovigilance-practices-gvp-module-xvi-risk-minimisation-measures-rev-3\\_en.pdf](https://www.ema.europa.eu/en/documents/regulatory-procedural-guideline/guideline-good-pharmacovigilance-practices-gvp-module-xvi-risk-minimisation-measures-rev-3_en.pdf)  
224 13. International Network for Epidemiology in Policy. INEP Position Statement Series  
225 Conflict-of-Interest and Disclosure in Epidemiology 2020 [Available from:  
226 <https://www.pharmacoepi.org/pub/?id=4149935D-0739-4AB3-4D78-B700BA0B2C2F>.  
227 14. Smith MY, Bahri P, Gaudino JA, Moreira RS, Danyluk GM, Palevsky SL. The role of  
228 epidemiologists in communicating SARS-CoV-2 evidence: a call for adopting standards. *Int J*  
229 *Epidemiol.* 2021;50(5):1410-5.
- 230 15. Wang SV, Schneeweiss S, Berger ML, Brown J, de Vries F, Douglas I, et al. Reporting to  
231 Improve Reproducibility and Facilitate Validity Assessment for Healthcare Database Studies  
232 V1.0. *Pharmacoepidemiol Drug Saf.* 2017;26(9):1018-32.
- 233 16. International Council for Harmonisation. ICH Harmonised Guideline: General Principles  
234 on Planning, Designing, Analysing, and Reporting of Non-interventional Studies That Utilise  
235 RealWorld Data for Safety Assessment of Medicines 2025 [Available from:  
236 [https://database.ich.org/sites/default/files/ICH\\_M14\\_Step4\\_Final\\_Guideline\\_2025\\_0905.pdf](https://database.ich.org/sites/default/files/ICH_M14_Step4_Final_Guideline_2025_0905.pdf).  
237 17. European Medicines Agency. Reflection paper on use of real-world data in non-4  
238 interventional studies to generate real-world evidence 2024 [Available from:  
239 [https://www.ema.europa.eu/en/documents/scientific-guideline/draft-reflection-paper-use-real-](https://www.ema.europa.eu/en/documents/scientific-guideline/draft-reflection-paper-use-real-world-data-non-interventional-studies-generate-real-world-evidence_en.pdf)  
240 [world-data-non-interventional-studies-generate-real-world-evidence\\_en.pdf](https://www.ema.europa.eu/en/documents/scientific-guideline/draft-reflection-paper-use-real-world-data-non-interventional-studies-generate-real-world-evidence_en.pdf).  
241 18. Centers for Medicare & Medicaid Services. Proposed Guidance Document: Study  
242 Protocols That Use Real-world Data 2025 [Available from: [https://www.cms.gov/medicare-](https://www.cms.gov/medicare-coverage-database/view/medicare-coverage-document.aspx?mcdid=39&doctype=all&timeframe=30&sortBy=updated&bc=21#_RWD)  
243 [coverage-database/view/medicare-coverage-](https://www.cms.gov/medicare-coverage-database/view/medicare-coverage-document.aspx?mcdid=39&doctype=all&timeframe=30&sortBy=updated&bc=21#_RWD)  
244 [document.aspx?mcdid=39&doctype=all&timeframe=30&sortBy=updated&bc=21#\\_RWD](https://www.cms.gov/medicare-coverage-database/view/medicare-coverage-document.aspx?mcdid=39&doctype=all&timeframe=30&sortBy=updated&bc=21#_RWD).  
245 [\\_RWD](https://www.cms.gov/medicare-coverage-database/view/medicare-coverage-document.aspx?mcdid=39&doctype=all&timeframe=30&sortBy=updated&bc=21#_RWD).  
246 19. US Food and Drug Administration. Considerations for the Use of Artificial Intelligence to  
247 Support Regulatory Decision-Making for Drug and Biological Products Guidance for Industry and  
248 Other Interested Parties 2025 [Available from: <https://www.fda.gov/media/184830/download>.  
249 20. US Food and Drug Administration. Study of Sex Differences in the Clinical Evaluation of  
250 Medical Products Guidance for Industry 2025 [Available from:  
251 <https://www.fda.gov/media/184907/download>.  
252 21. US Food and Drug Administration. Incorporating Voluntary Patient Preference  
253 Information over the Total Product Life Cycle: Draft Guidance for Industry, Food and Drug  
254 Administration Staff, and Other Interested Parties 2024 [Available from:

255 [https://www.fda.gov/regulatory-information/search-fda-guidance-documents/incorporating-](https://www.fda.gov/regulatory-information/search-fda-guidance-documents/incorporating-voluntary-patient-preference-information-over-total-product-life-cycle)  
256 [voluntary-patient-preference-information-over-total-product-life-cycle.](https://www.fda.gov/regulatory-information/search-fda-guidance-documents/incorporating-voluntary-patient-preference-information-over-total-product-life-cycle)

257