GSK U.S. Public Policy Position Paper
Communicating Comparative Effectiveness Research and Real World Evidence with Population Health Decision Makers

The Issue
Many healthcare and research organizations generate and use evidence beyond what is typically required for product approval, including comparative effectiveness research (CER) and real world evidence (RWE). As the growing electronic data infrastructure improves access to data, policymakers and healthcare purchasers continue to explore ways to achieve high-value, high-quality healthcare to inform population health decision making. CER and RWE help to fill an “evidence gap” by providing decision makers with more comprehensive information to supplement Randomized Clinical Trial (RCT) data to determine which treatment options are best for patients and ensure access to innovative, high-quality care.

The biopharmaceutical industry generates a significant amount of high-quality data that adheres to well-accepted research standards. Through the medicine discovery and development process, the biopharmaceutical industry acquires deep expertise that can provide a greater understanding of a disease, its treatment patterns and affect on patients, as well as product performance, to meaningfully contribute to population health decision making. However, the current regulatory environment does not allow for effective communication of this data.

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<th>Research Type</th>
<th>Definition</th>
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<td>CER</td>
<td>Comparative effectiveness research is comparative by design, and evaluates the benefits and harms of alternative methods to prevent, diagnose, treat, and monitor a clinical condition or to improve the delivery of care.</td>
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<td>RWE</td>
<td>Real world evidence is derived from Real World Data via analytics, observational studies, and pragmatic trials, assesses outcomes such as clinical, economic, and patient-reported. It seeks to include data that is collected outside of an RCT that is more generalizable to a “real-world” setting; e.g. patient registries, administrative claims databases, surveys, and medical records.</td>
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**Population health decision makers** include government agencies, payers, healthcare systems, guideline developers, compendia publishers, hospital pharmacy & therapeutics committees, and group purchasing organizations.

GSK Position
We believe that proactive communication of CER and RWE by healthcare stakeholders, including the biopharmaceutical industry, can significantly benefit public health by providing population health decision makers with an expanded evidentiary base to match the right treatment with the right patient. However, regulatory guidance is needed to define uniform methodology standards for comparative effectiveness and real world research that can be appropriately shared with population health decision makers.
**Key Points**

- Currently, there is a lack of clear regulatory standards for defining, generating and proactively communicating CER and RWE to population health decision makers. GSK urges the Food and Drug Administration (FDA) to provide guidance on when, how, and to whom industry is permitted to proactively share CER and RWE information.
- Timely and accurate information from comparative, observational, and/or retrospective studies, in addition to traditional RCT data, can help providers, payers, and patients make more informed treatment decisions and ultimately improve patient outcomes.
- The development of consensus standards by the FDA (using a multi-stakeholder body) for generating CER and RWE data ensures all healthcare stakeholders are held to equally rigorous measures to help ensure soundness of evidence.
- An environment that promotes collaboration through the appropriate sharing of accurate, science-based information enhances access to data and better informs conversations to ensure quality outcomes in an evolving value-based healthcare system.

**Evidence Communication Policy Opportunities**

- **Advocate** through the House Energy & Commerce Committee’s 21st Century Cures initiative for access to data to drive innovation (FDA Sentinel, PCORNet, CMS data, NIH Collaboratory Network, etc.), as well as legislation and regulation that encourages adoption of health information technology (HIT) and interoperability of data sources to increase the capacity and use of real world data.
- **Collaborate** with the Pharmaceutical Research and Manufacturers of America (PhRMA) and the Biotechnology Industry (BIO) to support increased regulatory clarity concerning communication of CER and RWE to payers, formulary committees, or other similar entities.
- **Advance** the work of the Medical Information Working Group (MIWG), a coalition of medical product manufacturers, to consider issues relating to the federal government’s regulation of truthful, non-misleading, scientifically substantiated manufacturer communications about new uses of approved drugs and approved/cleared medical devices.

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3. Drug approval requires safety and effectiveness data, where a drug’s effectiveness is established by “substantial evidence” defined in Section 505(d) of the Federal Food, Drug and Cosmetic Act (FFDCA) as “consisting of adequate and well-controlled investigations”. Generally at least two adequate and well-controlled studies are required to establish effectiveness. Congress amended the Act to clarify that FDA may consider data from one study and confirmatory evidence to constitute substantial evidence if FDA determines that such evidence is sufficient to establish effectiveness.