ISSUE SPOTLIGHT

The State of Real-World Evidence

Health care stakeholders are enthusiastic to explore whether real-world data sources (RWD), and the evidence they produce (real-world evidence (RWE), can inform and/or hasten medical product development and labeling. Importantly, RWE informs us about patient and provider choices as well as patient outcomes, which helps us understand whether medical products perform as intended in routine care. Ultimately, RWE may drive learning health systems that benefit patients by enhancing precision, equity, and care impact. To guide high-priority efforts aimed at improving the development and use of RWE, Duke-Margolis developed the Real-World Evidence (RWE) Collaborative. With over 40 members, the Collaborative has produced extensive analysis and insight relevant to the FDA's congressionally mandated mission to engage industry, academic, and patient stakeholders with the goal of better translating RWD into RWE to support their regulatory decisions. Duke-Margolis has become a trusted policy development resource for international regulators (e.g., the European Medicines Agency) to advance the use of RWE in product claims and labels related to safety, effectiveness, and comparative effectiveness. Duke-Margolis is also engaging Institute faculty, researchers, students, and external collaborators in RWE policy research that will advance RWE implementation and examine its ethical, legal, and social implications.

RWE policy stakeholders also face the challenge of understanding and communicating ethical, legal, and social implications to collecting and using various sources of RWD to generate RWE that can inform health care practice standards, medical product development and labeling, and patient care. A skilled workforce is required today and into the foreseeable future to address these challenges. Therefore, a three-pronged approach is taken at Duke-Margolis to engage Institute faculty, researchers, students, and external collaborators in RWE policy research:

The RWE Collaborative's latest work to implement this strategic plan includes the development of a new, Tableau-based International Harmonization Dashboard that is publicly available on the Duke-Margolis website. The webpage will be regularly updated to help internationally-focused RWE policy stakeholders track relevant and timely regulatory guidance, frameworks, international harmonization documents, and more.

Duke-Margolis Real-World Evidence Policy Portfolio

Engage RWE members and key stakeholders to develop practical insights and recommendations for RWE to:

- be a primary basis for regulatory decision-making
- support reasonable, equitable, and necessary coverage
- integrate research into routine care

Examine ELSI factors within the RWD lifecycle:

- Data bias, privacy, provenance/lineage & governance
- Informed consent, trust & transparency
- Beneficence and justice

Offer new and ongoing education opportunities for:

- Undergraduates
- Graduates
- Post-docs
- Bass Connection project teams

Advance Our Work

With additional support, the Duke-Margolis RWE policy research team could dedicate additional time and personnel to core academic activities. This includes but is not limited to more robust student, postdoc, scholar, and faculty engagement in timely RWE policy research education, hands-on training, and thought collaboration. Additional funding and support also would expand the team's capacity to facilitate engagement with patient groups and local communities impacted by or with a demonstrated interest in RWE policy. Our team's endeavors to conduct research activities would benefit from in-house technical support for timely data analysis and visualization, and an expansion of academically independent research focused on addressing ethical, legal, social, and policy implications to RWE implementation.