

Duke-Margolis Center for Health Policy 2023-2027 Strategy to Advance Real-World Evidence Policy

Since 2018, the Duke-Margolis Center for Health Policy's [Real-World Evidence \(RWE\) Collaborative](#) has convened leaders representing medical product developers, data companies, payers, research groups, providers, patient networks, as well as experts on regulatory affairs, law, health and data science, and policy, with a shared goal and interest to advance RWE policy. The RWE Collaborative has been described at global forums as a home for "[communities of practice](#)" that share tacit knowledge to problem solve and address, collectively, emergent challenges and opportunities within the scope and practice of RWD/RWE generation and use. Importantly, the RWE Collaborative's work has drawn interest among a growing list of key governmental stakeholders, both nationally and internationally.

In 2023, the RWE Collaborative Advisory Board developed and finalized the following five-year strategic plan to achieve its overarching mission and advance four key areas of RWE policy. Altogether, this plan will steer the RWE Collaborative's mission towards the implementation of best practices in the development, use, and application of real-world data and evidence to share learnings among industry, regulator, payer, and health system stakeholders to address their needs, improve the lives of patients, and drive improvements in care quality.

Duke-Margolis RWE Collaborative's Five-Year Strategic Plan (2023-2027)



FUNDING

DATA

METHODS

ENDPOINTS

RWE that routinely informs regulatory decision making.

Medical product regulators, including the US FDA and European Medicines Agency (EMA), increasingly consider RWD/E in applications submitted by clinical trial sponsors to support assessments of medical product safety and efficacy. Thus, advancing the acceptability of RWE to demonstrate medical product safety and efficacy among regulatory agencies is an ongoing mission for both the Center and the RWE Collaborative. Regulators like the [US FDA](#) and [European Medicines Agency](#) continue to release draft and final guidance on both broad and specific considerations for RWD/E, including but not limited

to considerations regarding data submission standards, data sources, data quality, study designs involving the use of RWD as a primary source of evidence, and international harmonization of RWD/E standards. Duke-Margolis has and will continue to engage, in a pre-competitive space and collaborative environment, its Advisory Group, members of the Regulatory Acceptability of RWE workstream, and other key stakeholders to directly inform national and international regulatory agencies engaged in advancing the state of RWE policy.

RWE that supports reasonable and necessary payer coverage.

Health insurance payers, both public and private, increasingly rely on RWD/E studies to make reasonable and necessary coverage determinations for new and existing medical products with demonstrated safety and efficacy across patient populations and subpopulations. Large national payers like the [US Centers for Medicare and Medicaid Services](#), in fact, encourage the development of innovative medical products and support the establishment of predictable and transparent evidence to support

accelerated beneficiary access to such products. Since the development and release of its 2022 white paper, entitled "[Aligning Shared Evidentiary Needs Among Payers and Regulators for a Real-World Data Ecosystem](#)," Duke-Margolis and its RWE Collaborative are focused on developing strategies to ensure real-world data is capable of generating high-quality and compelling evidence that meets the reasonable and necessary coverage needs of patients, health systems, payers, and regulators.

RWE that fosters collaboration and harmonization across ecosystems including international agencies.

There is growing interest among medical product regulators globally in uses of RWD/E to demonstrate medical product safety and, more recently, efficacy/effectiveness (i.e., [International Conference on Harmonisation Reflection Paper](#) and [International Coalition of Medicines Regulatory Authorities Statement](#)). This has led to discussions around the need to harmonize key terms, determine quality standards, promote study/study design transparency, and converge on RWD/E

guidance and best practices across the global regulatory landscape. Duke-Margolis engages members of the RWE Collaborative to monitor this growing global RWE policy landscape and contribute thought leadership to challenging topics and questions posed by medical product regulators and policymakers globally concerning RWD/E implementation to support timely patient access to innovative treatments and treatment approaches.

RWE that integrates research into routine care.

As the demand for more rapid and efficient means of rigorous clinical evidence generated about medical products from diverse practice settings increases, so has interest in point-of-care (POC) clinical trials that leverage EHRs and other RWD sources to integrate clinical research into routine care. Although interest in and technical capabilities to support POC trials continues to rise, actual implementation of scaled, impactful POC platforms remains limited. Building on the 2022 white paper, "[Point-of-Care Clinical Trials: Integrating Research and Care Delivery](#)," Duke-Margolis continues to explore, with health system stakeholders within and outside of

the RWE Collaborative, the prospective and systematic collection of RWD to drive randomized clinical trial conduct at the point-of-care, particularly to improve post-market evidence that may offer meaningful insights for regulatory agencies, policymakers, payers, and providers. Likewise, there is interest in the ability to use available data in real-time to inform clinical decision making in the absence of clinical guidelines. The same tools and approaches that can enable point-of-care trials, may provide opportunities to interrogate data in near-real-time to inform care decisions at the patient level.