

Payer Survey Highlights Confusion About the Definition and Utility of Real World Evidence in Rare Disease

Amidst growing use of real-world evidence (RWE) in clinical research and regulatory review of treatments for rare diseases, Syneos Health surveyed payers in the U.S. and Europe to learn how they define RWE and how they value it, relative to other evidence, when making coverage and reimbursement decisions. The results of our survey, supported by in-depth interviews in a qualitative research phase, suggested that U.S. and European payers are only embracing RWE *within limited parameters* when it comes to rare-disease coverage decisions.

The survey responses reflected significant confusion around terms such as “real-world evidence” and “real-world data.” Furthermore, many payers indicated they are not receptive to RWE-based representations or health economic forecasts in sponsors’ dossiers. Potentially, this perception gap may have adverse consequences when it comes to market access for new treatments—especially in rare diseases where there is high unmet need.

While the lag in payers’ understanding of RWE has not emerged as a significant market access barrier at the present time, the rapid pace of technological innovation threatens to widen the gap. This could lead to coverage and reimbursement hurdles in the future, as innovations such as “virtual” clinical trials and N-of-1 studies become more common. Also, budget constraints for Medicaid, and to a lesser extent commercial payers, will put pressure on reimbursement negotiations for higher-priced orphan drugs, many of which are approved with single-arm trials using real-world data. Further, value-based agreements may become more important for getting coverage amidst budget constraints as more transformative therapies come to market – but those agreements typically rely on RWE to tell us whether the product is working for patients covered under the agreement.

The survey results suggest that industry-wide educational initiatives focused on RWE could help narrow the perception gap between regulators and manufacturers, on one side, and payers on the other. Through well-structured education exercises, standards setting, and fact-finding missions on the frontiers of evidence sciences, we believe that stakeholders can collaborate to close the knowledge gaps on behalf of patients with rare diseases.