Real-World Effectiveness of Immunotherapy in Hematologic Malignancies: Evidence from Big Data

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Immunotherapies—including monoclonal antibodies, antibody–drug conjugates, bispecific T-cell engagers, immune checkpoint inhibitors, and cellular therapies such as CAR-T—are reshaping hematologic oncology, yet pivotal trials frequently enroll selected populations and can lag behind rapidly evolving standards of care. We applied big data analytics to generate real-world evidence (RWE) by integrating multi-source routinely collected datasets, including electronic medical records, institutional and disease registries, laboratory information systems, and claims/administrative data, to construct longitudinal cohorts across major hematologic malignancies (e.g., lymphoma, leukemia, and plasma cell disorders) and across lines of therapy. Reproducible algorithms were implemented to define treatment exposure, sequencing, and lines of therapy, while outcomes captured effectiveness (response proxies and time-to-event endpoints such as time-to-next treatment and overall survival), safety deliverability (hospitalization, serious infections, immune-mediated toxicities, and real-world dose modification/discontinuation), and healthcare resource utilization and costs. Comparative analyses used propensity-score methods and time-to-event modeling to benchmark emerging immunotherapies against contemporary real-world standard-of-care comparators and to quantify heterogeneity of treatment effects across clinically relevant subgroups underrepresented in trials. Across disease settings, we observed substantial gaps between trial eligibility and routine-care populations, highlighting limits to generalizability of trial-only evidence. RWE provided actionable insights into real-world effectiveness, confirmed that key immunotherapy toxicities are often manageable with structured monitoring and early-intervention pathways, and enabled timely identification of strategies with limited incremental value in practice. Linking outcomes to observed resource use supported standardized, decision-ready assessments of economic burden and value. Overall, big data-derived RWE complements RCTs by delivering timely, practice-grounded evidence to guide immunotherapy prioritization, sequencing, implementation, and policy decisions in hematologic malignancies.