Characterizing the Contribution of RWE in Healthcare Decision-Making in Oncology

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What we learned

A decade of progress and increased RWE integration across regulatory, HTA, and clinical practice

RWE-related publications in oncology have increased **1.5-fold** in the last decade, underscoring its rising importance in healthcare decisions and the need for rigorous standards and guidance in RWD collection and evidence dissemination (**Figure 2**).

Introduction

- Real-world evidence (RWE), derived from real-world data (RWD), complements gold standard randomized controlled trial (RCT) findings by capturing outcomes in broader, more diverse populations (**Figure 1**)[1-3]
- In oncology, treatments are often tailored to small, heterogenous patient populations, and the therapeutic landscape is constantly evolving[4]
- RWE in oncology can provide valuable insights into real-world effectiveness and safety, economic and humanistic implications, and regulatory and reimbursement decisions[1,5]

Objective

- Characterize RWE generation in oncology and its growing role in healthcare decision-making over the last decade

Figure 1: RCT and RWE attributes[2,6]

	RCT	RWE
Purpose	Specifically addresses efficacy and safety	Multipurpose, pertinent to healthcare decision-making
Type/Design	Interventional (randomized); prospective	Observational (non-randomized); prospective/retrospective
Treatment/Follow-up	Designed, protocol-driven	In actual practice
Patient population/Setting	Homogenous, narrow, and restricted in a controlled setting	Heterogenous, large, and unrestricted in a real-world setting
Comparator	Placebo or select alternative interventions	Multiple alternative interventions
Patient monitoring	Per protocol	Variable
Attending physician	Study investigator	Multiple practitioners
Application	Regulatory drug approvals through safety and efficacy studies	Post-marketing surveillance, healthcare decision-making including regulatory approvals and reimbursement
Limitation	Lack of generalizability, low external validity	Potential for bias, low internal validity

Methods

Design	Targeted literature review using PubMed
Scope	Peer-reviewed oncology-related publications from January 01, 2015 to December 31, 2024
Search Strategy	Search terms related to cancer, RWE, regulatory, health technology assessment (HTA)/ reimbursement, real-world effectiveness and safety, adherence, treatment patterns, economic outcomes, humanistic outcomes were utilized

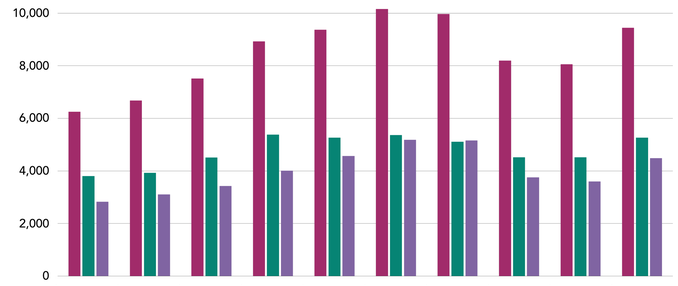
Results

Figure 2: Trends in RWE-related publications in oncology

Total RWE publications
1.5x ↑
6,255 in 2015 → 9,447 in 2024

Non-observational publications
1.4x ↑
3,804 in 2015 → 5,269 in 2024

Observational publications
1.6x ↑
2,831 in 2015 → 4,448 in 2024



	2015	2016	2017	2018	2019	2020	2021	2022	2023	2024
Total (N=84,577)	6,255	6,677	7,513	8,931	9,370	10,160	9,969	8,196	8,059	9,447
Non-observational (n=47,678)	3,804	3,930	4,513	5,379	5,269	5,367	5,107	4,520	4,520	5,269
Observational (n=40,129)	2,831	3,105	3,429	4,013	4,568	5,183	5,157	3,755	3,600	4,488

Trends in non-observational RWE publications

Regulatory
2.0x ↑
2,558 total publications
150 in 2015 → 305 in 2024

HTA/Reimbursement
1.9x ↑
579 total publications
46 in 2015 → 89 in 2024

Non-observational: Regulatory guidance, HTA frameworks, systematic reviews, commentaries, and policy papers discussing the role and application of RWE in decision-making, rather than presenting original data analyses.

Growing use of RWE in regulatory and reimbursement decisions signals its rising credibility

Trends in observational RWE publications

Effectiveness and safety
2.8x ↑
6,239 total publications
346 in 2015 → 961 in 2024

Humanistic
2.2x ↑
3,210 total publications
192 in 2015 → 418 in 2024

Economic
1.3x ↑
2,635 total publications
192 in 2015 → 242 in 2024

Adherence
1.4x ↑
2,148 total publications
162 in 2015 → 227 in 2024

Treatment patterns
4.1x ↑
865 total publications
33 in 2015 → 136 in 2024

Observational: Analyses using data sources such as electronic health records (EHRs), registries, and claims databases to examine effectiveness, safety, humanistic, economic, and treatment patterns outcomes in real-world settings.

Rise in observational studies highlights growing role of RWDs in complementing RCTs

Total publications represents the sum of studies between 2015-2024 in that category.

Discussion

- The marked increase in RWE-focused publications over the past decade underscores the expanding recognition of RWD as a vital complement to RCTs in oncology decision-making
- Recent surveys indicate 90% of payer decision-makers globally report that RWE can support reassessment by demonstrating long-term effectiveness, while 80% value RWE for addressing post-market safety assessments[7]

Data Availability and Infrastructure

- Expansion of diverse RWD sources - EHRs (e.g., Flatiron), claims (e.g., IQVIA), registries (e.g., CancerLinQ), and digital health tools - enables outcome assessment in broader, underrepresented populations and real-world settings where RCT data may be limited[8,9]
- Linked datasets support robust longitudinal analyses, though variability in data capture and quality necessitates rigorous documentation and fit-for-purpose evaluation[10]

Methodological Standards and Rigor

- Adoption of best practices and frameworks like STaRT-RWE and ISPOR/ISPE guidance, and protocol registration promotes transparency, reduces bias, and enhances reproducibility and credibility[11-13]
- Effective RWE dissemination requires translating complex analyses into clear, actionable insights for regulators, payers, and clinicians
- Clear documentation of data provenance and attention to confounding, temporality, and missingness ensures credibility[14]

Regulatory and Reimbursement Frameworks

- Regulatory and HTA agencies have formalized RWE into oncology decision-making (**Figure 3**)[15]
- FDA's Advancing RWE Program and EMA's DARWIN EU are leading examples, with similar guidance emerging globally (MHRA [UK], TFDA [Taiwan], Health Canada, NMPA [China], and PMDA [Japan])[15]
- Recent analyses show a growing use of RWE in decision-making: EMA referenced RWE in 31% of oncology assessments (2020-2022), with uptake increasing over time[16]

Figure 3: Regulatory and HTA Frameworks[15,17]

Food and Drug Administration (FDA)

2016	2018	2020	2021	2022	2023	2024
– 21st Century Cures Act – Initiated RWE considerations	– RWE Program Framework – EHR use in clinical investigations		– Cures 2.0 Act (draft): Proposed RWE task force and FDA approval support – Guidance on RWD standards	– Guidance on submitting RWD/ RWE to FDA for drugs and biologics – Advancing RWE program to support new labeling claims and post-approval study requirements	– Considerations for RWD/RWE use in regulatory decision-making – Registry-based data guidance in regulatory decision-making	– EHRs/medical claims data to support regulatory decision-making – Integrating RCTs into routine clinical practice
	– NICE – Evidence standards framework for digital health technologies	– EMA 2025 strategy – Big Data Task Force recommendations	– Registry-based study guidelines – MHRA RWD-RCT guidance to support regulatory decisions	– DARWIN EU launch – Guidance on metadata catalog of RWD sources – Data quality framework for EU medicines regulation		– RWE framework to support EU regulatory decision-making

European Medicines Agency (EMA)

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Envisioning the Future of RWE

RWE as a Regulatory Standard

RWE can support regulatory decision-making in initial approvals, label expansions, and new indications

Open and Integrated RWD

High-quality RWE based on standardized, inter-operable data systems, with accessible and linkable RWD can facilitate continuous evidence generation across healthcare settings

Scaling Outcome-Based Care

RWE-driven value-based healthcare can enable outcome-based contracts where pricing reflects real-world performance, promoting sustainability and patient-centered value

Global Harmonization of RWE Standards

Globally aligned RWE frameworks can ensure consistent, transparent evidence, supporting international HTAs and policy decisions

RWE at the Point of Care

RWE insights integrated into clinical decision support systems can provide real-time, value-based guidance to improve clinical decision-making