Study Design Trends in Registry-Based Oncology Studies: Analysis of HMA-EMA Real-World Data Catalogues

RWD175

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INTRODUCTION

- Designing oncology RWE studies requires meticulous alignment of endpoints, patient populations, and clinical care pathways to account for real-world variability and ensure methodological robustness.
- The intrinsic heterogeneity of tumors, rapid evolution of targeted therapies, and molecular stratification amplify the complexity of generating reliable real-world oncology evidence.
- Fragmented data ecosystems spanning registries, hospital EHRs, and payer databases impede longitudinal linkage, limiting visibility into patient journeys and treatment outcomes.
- High-quality, interoperable data repositories with standardized coding and traceable provenance are critical to generating regulatory-grade, high-fidelity oncology RWE.
- The **EMA–HMA RWD Catalogue** integrates diverse European oncology data assets, streamlining the identification of fit-for-purpose registries and aligning data infrastructure with evolving regulatory evidence needs.

OBJECTIVE

This study aimed to characterize registry-based oncology studies captured in the EMA-HMA RWD catalogues, focusing on data source selection, design features, and regulatory alignment.

METHODS

Study Identification

- A **structured descriptive framework** was applied to the EMA–HMA RWD catalogues to characterize registry-based oncology studies.
- Included studies were **non-interventional and oncology-focused**, using registries as primary or linked data sources.
- A **two-stage curation process** excluded duplicates and non-oncology entries, ensuring analytical consistency.

Data Processing & Quality Control

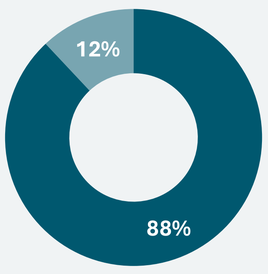
- Extracted study parameters across **standardized metadata domains** such as cancer type, geography, regulatory intent, and data provenance.
- **Data provenance** categorized as *secondary* (standalone registries) or *linked* (registry–claims/EHR integrations), reflecting interoperability maturity.
- A **dual-review QC process** ensured data accuracy, reproducibility, and transparent adjudication of discrepancies.

Analysis Framework

- Applied **descriptive synthesis** using frequency and proportion metrics to summarize study features.
- Categorized objectives into **epidemiology, safety, effectiveness,** and **drug utilization** domains.
- Maintained **transparency and traceability** through standardized abstraction matrices and controlled documentation.

RESULTS

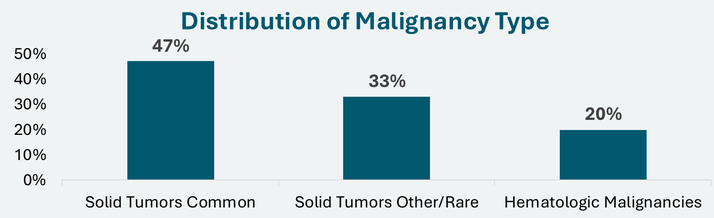
Data Sources in Oncology Real-World Evidence Studies



88%
Secondary Data
Registry or HER-based studies dominate

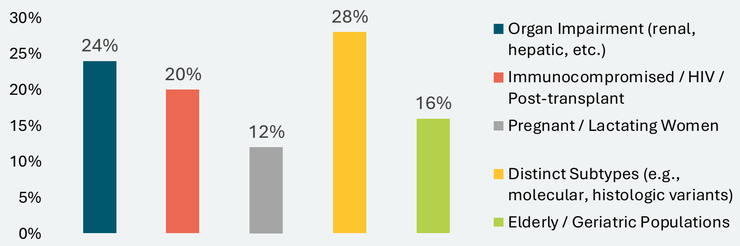
12%
Linked Datasets
Integrated multi-source data for validation

Distribution of Malignancy Type



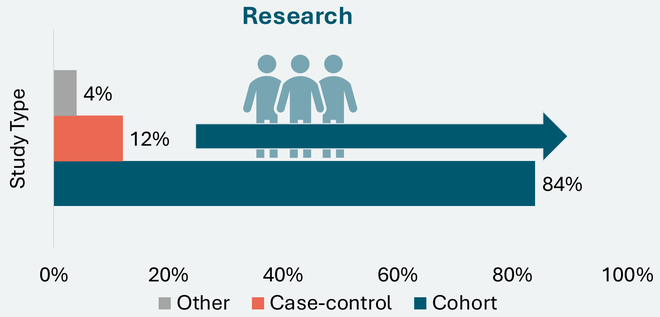
Almost half targeted common solid tumors, while others explored blood cancers and rarer solid malignancies.

Focus on Special and Underrepresented Populations



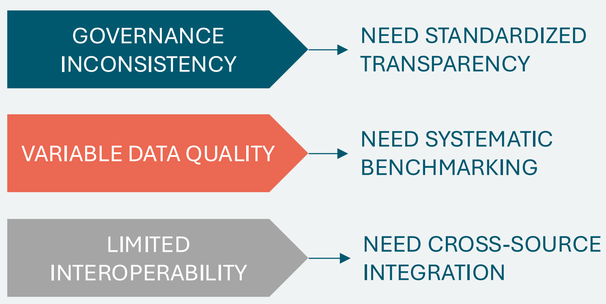
This focus aligns with the push for **inclusive evidence generation** in oncology.

Cohort Studies Dominate RWD Oncology Research



This indicates a preference for **longitudinal patient follow-up** and outcome tracking.

Gaps and Opportunities in HMA-EMA RWD Catalogues



Purpose of RWD Oncology Studies

24%
Misc (Quality of Life, Care Patterns, etc.)

21%
Epidemiology and Safety

17%
Drug Utilization and Effectiveness

Population Insights from Oncology RWD Catalogues

- **RWD data catalogues enable population realism**, capturing age, comorbidity, and treatment diversity often excluded from clinical trials—helping early designs align with actual care settings and patient variability.
- **Through registry-linked population mapping**, catalogues reveal actionable subgroup patterns—such as molecular, demographic, and clinical stratifiers—that strengthen **evidence-driven cohort selection** in early oncology development.

Design Insights from Oncology RWD Catalogues

- **Intervention Focus**: Nearly 70% target oncology drugs or regimens, while about 20% assess cancer risk from non-oncology agents, signaling expanding pharmacovigilance applications.
- **Comparators & Analytical Rigor**: >70% of comparative studies use propensity-score or Cox-based models; control definitions rely on treatment-exposed vs. non-exposed cohorts rather than placebo arms.

Outcome insights from Oncology RWD Catalogues:

- **Overall survival (OS)** dominates outcome selection, featuring in >60% of studies—reinforcing its position as the real-world benchmark for long-term cancer effectiveness.
- **Epidemiologic endpoints** such as incidence, prevalence, and mortality appear in >30% of studies, underscoring RWD's strength in mapping disease burden and survival trends.
- **Progression-related outcomes (PFS/DFS)** are notable but secondary, reflecting a balanced focus between disease control and real-world treatment trajectories.
- **Treatment pattern analyses** (>20% of studies) highlight how RWD informs real-world therapy utilization, sequencing, and adherence.
- **Safety and molecular outcomes** remain underrepresented (<20% and <10%), signaling opportunity areas for integrating toxicity and biomarker data into future RWD designs.

Regulatory Alignment Insights from Oncology RWD Catalogues:

- Over **half (56%)** of all oncology RWD studies were conducted in support of an **EU Risk Management Plan**, showing strong regulatory alignment and post-authorization safety commitment.

Compliance Check insights from Oncology RWD Catalogues:

- **~45% ENCePP adherence highlights a growing foundation of governance practices** that can be leveraged to design oncology RWD studies with stronger regulatory and ethical alignment.
- Limited execution of full data conformance and stability checks emphasizes the ***opportunity to embed data quality–by–design principles***, enhancing reliability of early oncology endpoints.
- Partial CDM mapping (~35%) and modest data linkage (~25%) reveal where ***harmonized, multi-source integration can strengthen oncology study comparators and external control arms.***

Strengths

- Offers structured, transparent mapping of oncology RWD studies aligned with regulatory frameworks.
- Enables rapid identification of design trends and fit-for-purpose data sources for study planning.

Limitations

- Limited granularity restricts assessment of study quality and methodological rigor.
- Static, snapshot-style data reduce visibility of ongoing updates or emerging registries.

Actionable Insights

- Enhance catalogue interoperability and real-time data refresh to improve study feasibility assessments.
- Integrate standardized quality and governance metrics to support regulatory-ready oncology RWE design.

"Smooth submissions start with smart data."

"Transparency today, trust tomorrow."

"Better designs begin with standardized insights."

DISCUSSION

- The EMA–HMA RWD catalogue provides a unified view of oncology datasets across Europe, enabling registry mapping aligned with specific tumor types and evidence needs.
- Dataset-level details on population size, data quality, and cancer subtype help optimize feasibility assessments and real-world study design.
- Heterogeneity in data collection, coding, and outcome definitions underscores the need for harmonization and transparent analytic methods.
- Diverse access procedures, consent models, and governance structures can delay data acquisition, highlighting the value of early engagement with registry owners.
- Incorporating catalogue insights during protocol planning enhances registry selection, patient cohort targeting, and endpoint prioritization.
- Robust interpretation of catalogue outputs requires cross-functional expertise to ensure data use remains fit-for-purpose, reproducible, and methodologically sound.

CONCLUSION

- EMA-HMA RWD catalogue strengthens oncology study design by aligning research with real-world patient characteristics and treatment patterns.
- Thoughtful use of RWD insights in early design can enhance trial feasibility, support adaptive strategies, and foster patient-centric outcomes.

REFERENCES

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