Exploring the Potential of Real-World Data Sources in Oncology and Rare Diseases: A North American Perspective

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Background/Objectives

- ✓ Real-world data (RWD) sources can provide valuable insights into outcomes and costs of healthcare interventions.
- ✓ RWD acceptability by HTAs has increased significantly and presents a great opportunity for manufacturers to demonstrate the value of their products.
- ✓ However, availability and accessibility to these sources vary across different countries and there is a critical unmet need for a comprehensive repository of RWD sources to access patient outcomes and tailor healthcare interventions more effectively, especially in areas of oncology and rare diseases.

Objectives: This study aimed to review the current state and potential of RWD sources in North America [United States (USA), Canada and Mexico], with a focus on oncology and rare diseases.

Study Design

- ✓ We conducted a targeted review of articles published in PubMed, using keyword-based screening and snowball methodology in accordance with Preferred Reporting Item for Systematic Reviews and Meta-Analyses (PRISMA) guidelines.
- ✓ Population, Intervention & Comparators, Outcomes and Study design (PICOS)-based criteria was applied to identify articles on relevant RWD sources for North American oncology and rare disease populations discussed in Table 1 below.
- ✓ Information on database characteristics and access to these sources was extracted and a repository of these identified databases was created using Microsoft PowerBI™.

PICOS screening criteria

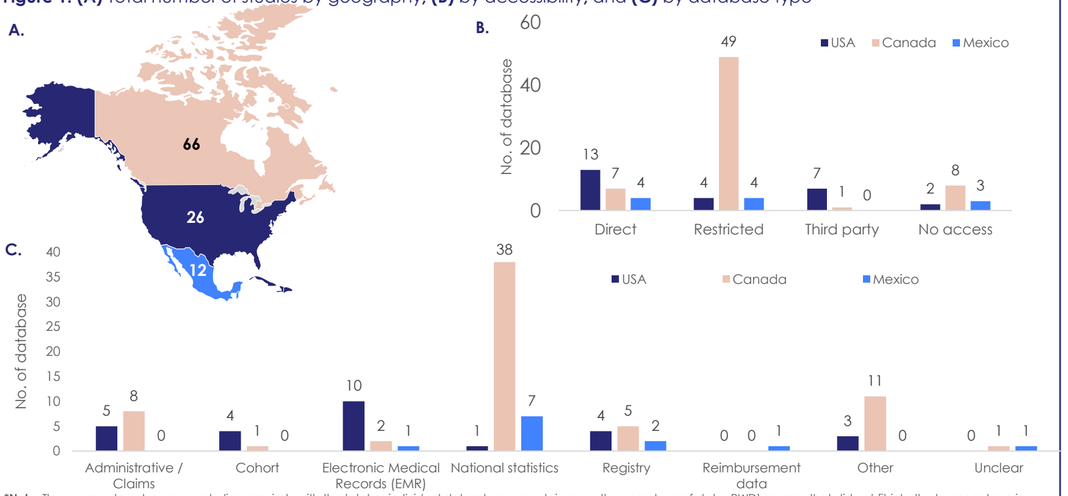
Table 1. Study selection criteria

PICOS	Inclusion criteria		Exclusion criteria	
Population	Oncology or rare diseases		Non-human population	
Interventions & comparators	No restrictions			
Outcomes	• Database related information • Type of outcome reported per database • Clinical outcomes • Economic outcomes	• Treatment outcomes • Patient-related outcomes • Laboratory • National statistics • Unit costs	Studies not including at least one of the outcomes listed in the inclusion criteria	
Study type	Real world evidence studies including: • Registry analyses • Database analyses • Epidemiological studies • SLRs, reviews, and meta-analyses *(for cross-checking only*)*		• Randomized controlled trials • Interventional studies • Case reports/case series	• Chart reviews • Editorials • Notes/ comments/ letters • Studies with <20 patients in the whole population
Language/Time	No restrictions			
Geography	USA, Canada, and Mexico		Studies outside of USA, Canada, and Mexico	

*Bibliographic screening from SLRs, reviews and meta-analyses was undertaken. The studies itself were excluded and relevant references from these studies were included (if any).

Findings

Figure 1. (A) Total number of studies by geography, **(B)** by accessibility, and **(C)** by database type*



***Note**: The aggregate values may not align precisely with the total as individual datasets may contain more than one type of data. RWD) sources that did not fit into the larger categories such as research institutes or consortiums are categorized as 'Other.' If publicly available information on a database was not accessible, it was classified under 'Unclear.'

Table 2. Characteristics of Data Sets with Top 5 Highest Patient Counts Across Countries

Database Name	Data Type	Entry date of the RWD: Accessibility	Entry date of the RWD: Last update	Entry date of the RWD: Creation date	Number of patients	Outcomes covered in the RWD: Demographic	Efficacy	Safety	Treatment patterns	Mortality	Laboratory	Comorbidities	Economic outcomes	Resource use	Patient reported outcomes
Surveillance, Epidemiology, and End Results Program (SEER)	Registry	Direct	2021	1973	18 M	■	■	■	■	■	■	■	■	■	■
Centers for Medicare & Medicaid Services (CMS)	Claims	Third party	2021	1965	45 M	■	■	■	■	■	■	■	■	■	■
Optum (OM registry)	Claims + EMR	Third party	NR	1993	100 M+	■	■	■	■	■	■	■	■	■	■
National Cancer Database (NCDB)	EMR	Direct	2017	2008	11 M+	■	■	■	■	■	■	■	■	■	■
Marketscan	Claims	Third party	2022	2012	273 M+	■	■	■	■	■	■	■	■	■	■
Canadian Cancer Registry	Registry	Restricted	2024	1992	NR	■	■	■	■	■	■	■	■	■	■
Ontario Cancer Registry	Registry	Restricted	2023	1964	NR	■	■	■	■	■	■	■	■	■	■
Registre québécois du cancer	Registry	Restricted	2023	1984	NR	■	■	■	■	■	■	■	■	■	■
British Columbia Cancer Registry	Registry	Restricted	2019	1969	NR	■	■	■	■	■	■	■	■	■	■
Alberta Cancer Registry	Registry	Restricted	NR	1942	NR	■	■	■	■	■	■	■	■	■	■
Catálogo universal de servicios de salud	Registry	Direct	2019	2008	NR	■	■	■	■	■	■	■	■	■	■
Costos Unitarios por Nivel de Atención Médica	Registry	Direct	2022	2015	NR	■	■	■	■	■	■	■	■	■	■
Instituto Nacional de Estadística y Geografía (INEGI)	Registry	Direct	2023	2012	126 M	■	■	■	■	■	■	■	■	■	■
Registro de Cancer en Ninios y Adolescentes	Registry	NR	2023	2005	NR	■	■	■	■	■	■	■	■	■	■
Registro Nacional de Cáncer en México	Registry	Restricted	2023	2018	15 M	■	■	■	■	■	■	■	■	■	■

■ Available ■ Not clearly reported ■ Not available

Abbreviations: CMS: Centers for Medicare & Medicaid Services; EMR: electronic medical record; INEGI: Instituto Nacional de Estadística y Geografía; M: million; MEX: Mexico; NCDB: National Cancer Database; NR: not reported; OM: Optum; RWD: real-world data; SEER: Surveillance, Epidemiology, and End Results Program; SD: standard deviation.

Summary Of Findings

- ✓ The 104 identified RWE sources (USA=26, Canada=66 and Mexico=12) fall into various categories: administrative/claims (n=71)†; registries (n=11); and electronic medical records (n=13).
- ✓ Information available in these databases includes demographic and clinical patient characteristics, health care utilization (HCRU) as well as quality of life (QoL).
- ✓ While Canada boasts a substantial amount RWD sources in oncology/rare diseases (n=66), the US has a greater number of databases that offer direct access (n=13) to information.

Note: †The aggregate values may not align precisely with the total as individual datasets may contain more than one type of data.

Discussion

- ✓ Real-world data (RWD) plays a crucial role in oncology and rare disease research, especially in situations where patient numbers in clinical trials are limited and post-disease progression treatment pathways are unclear. Gathering information on clinical outcomes, healthcare resource utilization (HCRU), quality of life (QoL), and other pertinent patient data can be challenging without the use of RWD.
- ✓ We identified 104 RWE databases for oncology and rare diseases, with varying accessibility.
- ✓ HTA authorities including FDA have been publishing guidance's for incorporating real-world evidence effectively in their evaluations.
- ✓ Effective utilization of these identified RWD sources could provide relatable insights and further healthcare research in oncology and rare diseases.

Limitations

- ✓ While identified datasets have been selected through targeted searches, the scope of our study is limited to oncology and rare diseases. Therefore, systematic searches might yield comprehensive evidence across other disease domains, including rare diseases.
- ✓ The current approach assumes that Canadian provinces with higher population have higher cancer prevalence.
- ✓ Relevant information about each data source was not always publicly available, therefore the availability of certain variables of interest could not be assessed.

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