AN INSIGHT INTO RWE STUDIES AS A COMPONENT OF SUBMISSIONS TO NICE & CDA IN THE LAST 3 YEARS

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RWE is increasingly recognized as a key component of HTA submissions and gained more acknowledgement across HTA agencies in recent years

Current Situation

RWE is increasingly valued for its insights into treatment effectiveness and safety

46 RWE-related documents from regulatory and HTA bodies differing significantly in scope and content

The lack of standardization creates challenges for manufacturers preparing multiple global submissions

This research explores the **growing trend of RWE submissions to NICE and CDA over the past 3 years** and **the potential impact of recent RWE guidance from these agencies**

NICE framework and CDA guidance stand out by offering a set of best practice standards for all phases of RWE development*

The NICE framework and CDA guidance were published recently and are designed to be regularly updated

Guidance for Reporting Real-World Evidence (2023)

- ✓ RWE decision-grade standards for regulatory or HTA submissions
- ✓ Aimed to harmonize RWE principles for both Canadian HTA agencies and regulators
- ✓ Includes nine recommendations focused on five strategic priority areas

NICE real-world evidence framework (2022)

- ✓ Best practices for planning, conducting, and reporting RWE
- ✓ Data Suitability Assessment Tool (DataSAT) for evaluating data quality and relevance

*All phases of RWE i.e. from planning and design to data reporting

Between 2021-2023, 52% (UK) and 37% (Canada) of medicines' HTAs included RWE studies, making them suitable for this research analysis

Methodology

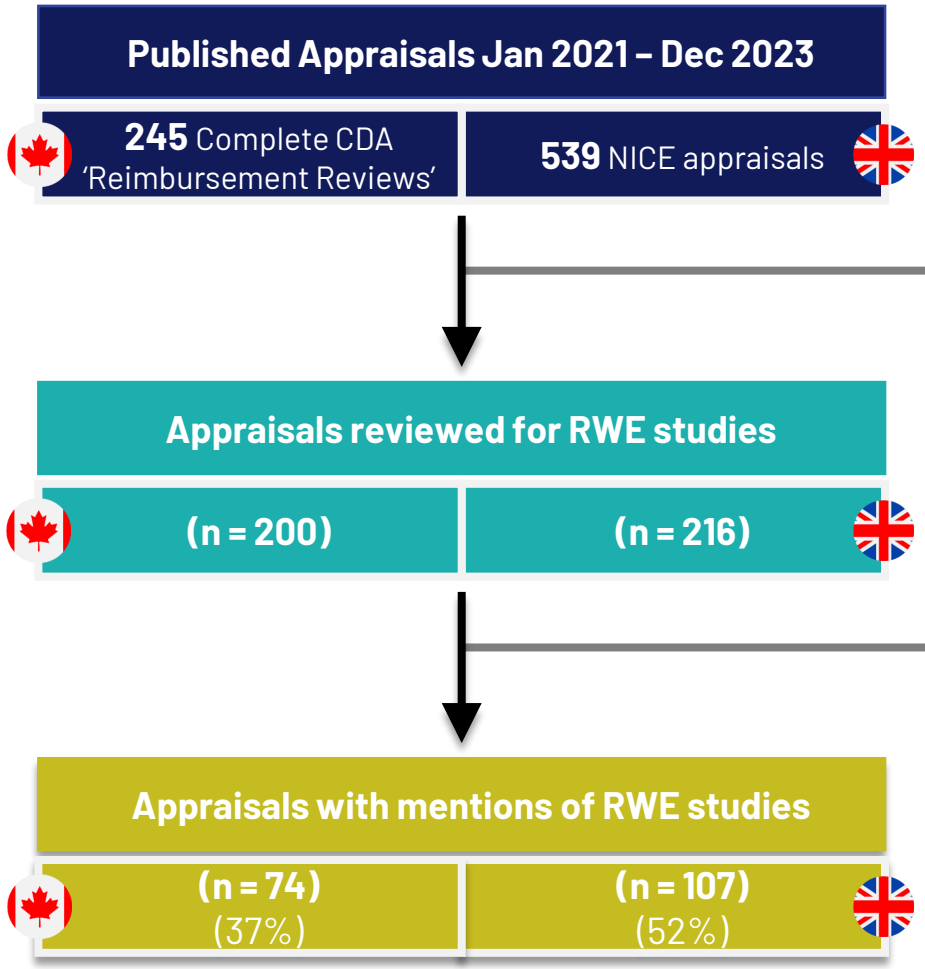
Review of HTA appraisals published in 2021-2023

Scope

HTA agencies in **Canada (CDA-AMC)** and **UK (NICE)**

Sample for analysis

74 HTA appraisals in Canada and **107 HTA appraisals in the UK**



Excluded Appraisals

Non-Relevant Appraisals

(n = 45)
Reimbursement algorithm update (n = 38)
Superseded by a new submission (n = 5)
Streamlined Drug Class/ Therapeutic review (n = 2)

(n = 323)
Terminated appraisals (n = 57),
Non-interventional Guidance
(n = 266)

Appraisals not mentioning RWE

(n = 126)

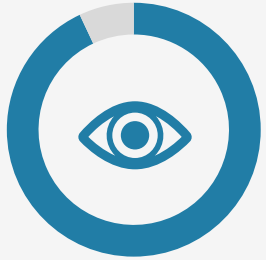
(n = 109)

Observational studies make up the majority of RWE submitted to both HTA agencies, typically employing a retrospective design

Type of RWE studies submitted

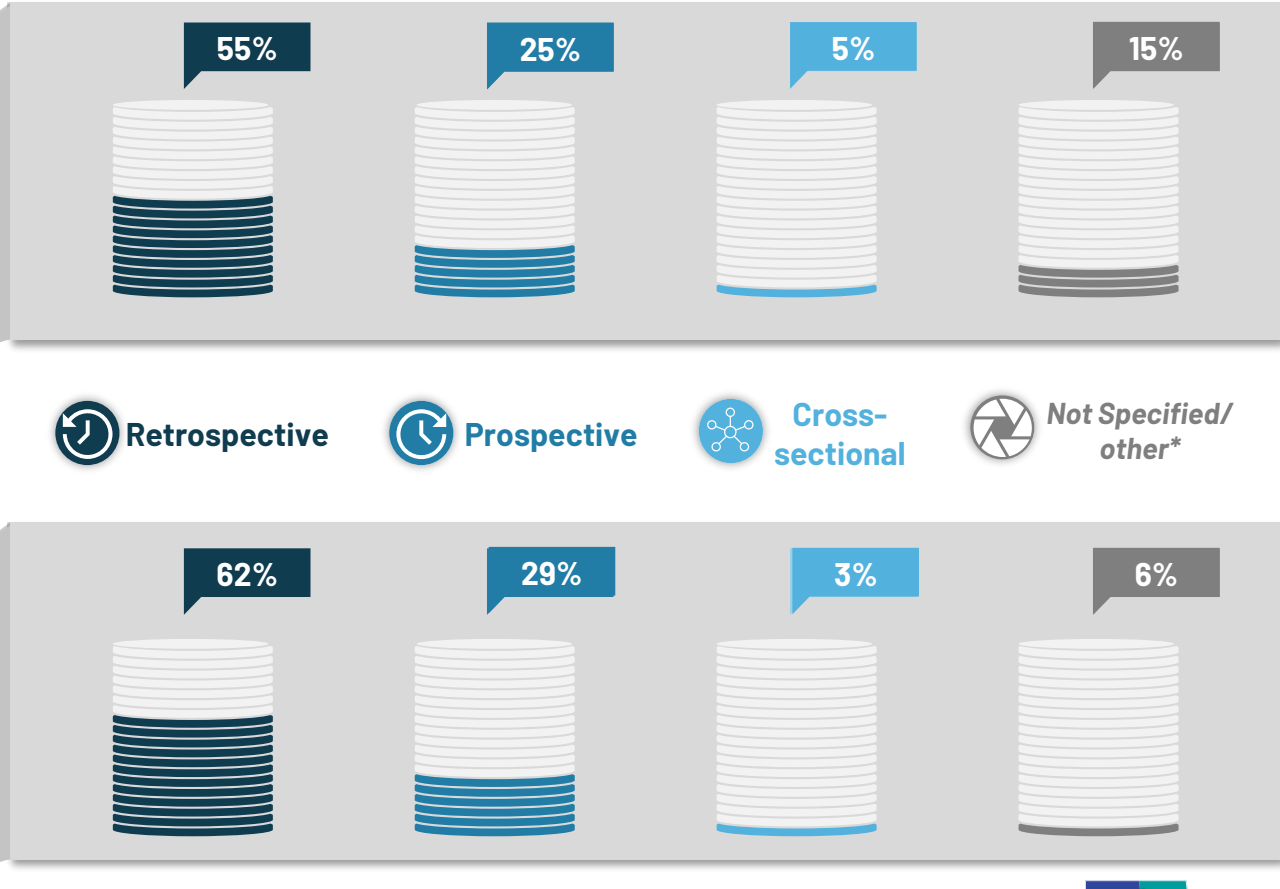
More than **80%** of submitted RWE studies were Observational

Observational Studies are considered systematic assessments of events, without interference, using routine data*, in contrast to other RWE studies such as pragmatic trials, surveys, interviews or focus groups*



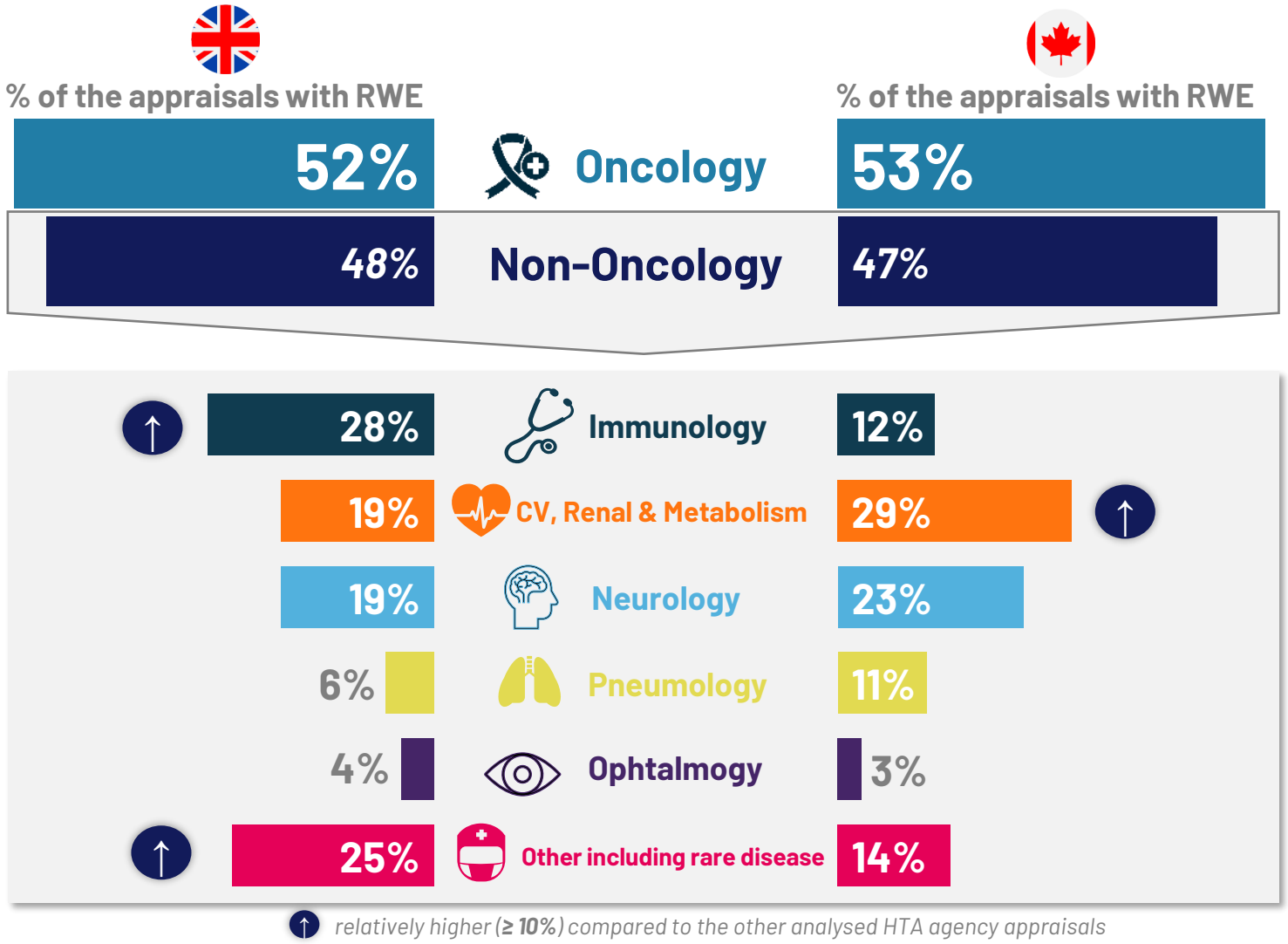
More than **93%** of submitted RWE studies were Observational

Time perspective of submitted observational studies



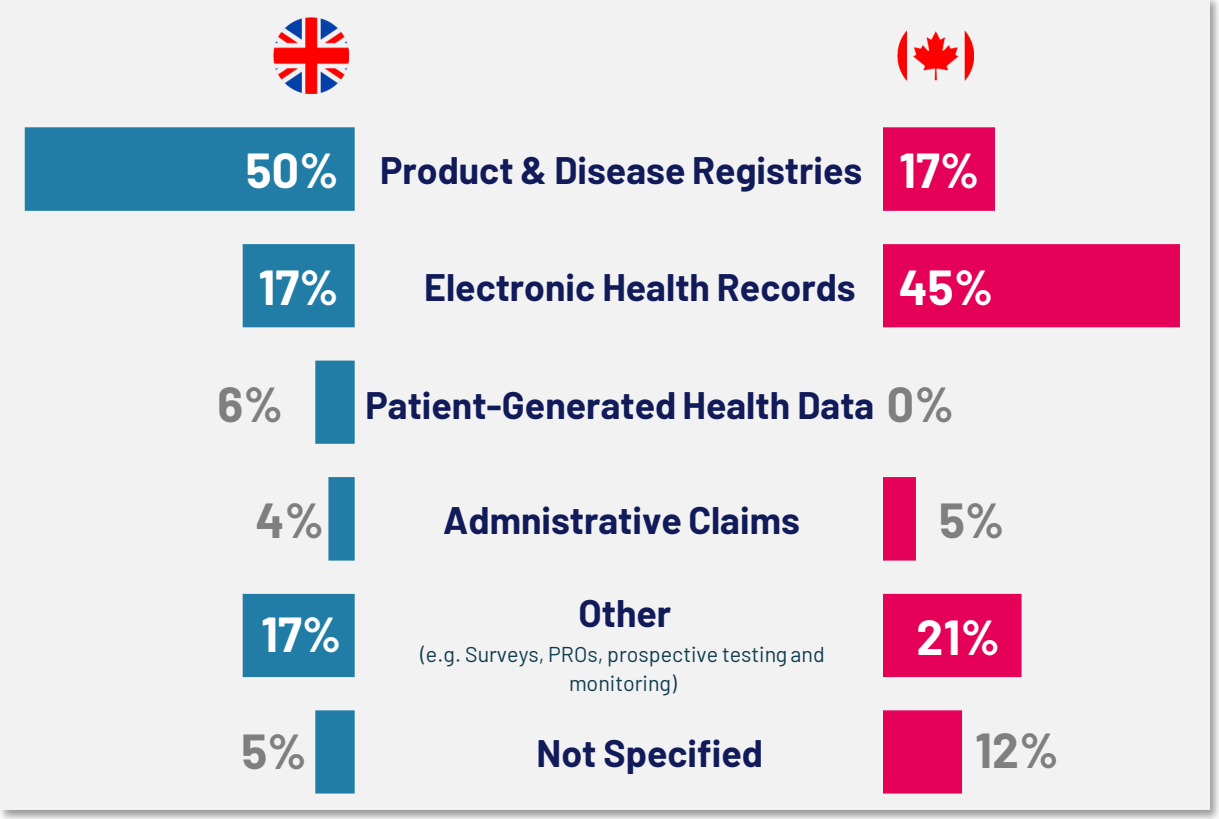
**Studies that analysed multiple RWE sources with unclear time frames and lacked a uniform retrospective or prospective design*

Oncology is the therapy area with the highest percentage of appraisals including RWE, showing consistency across the two HTA agencies

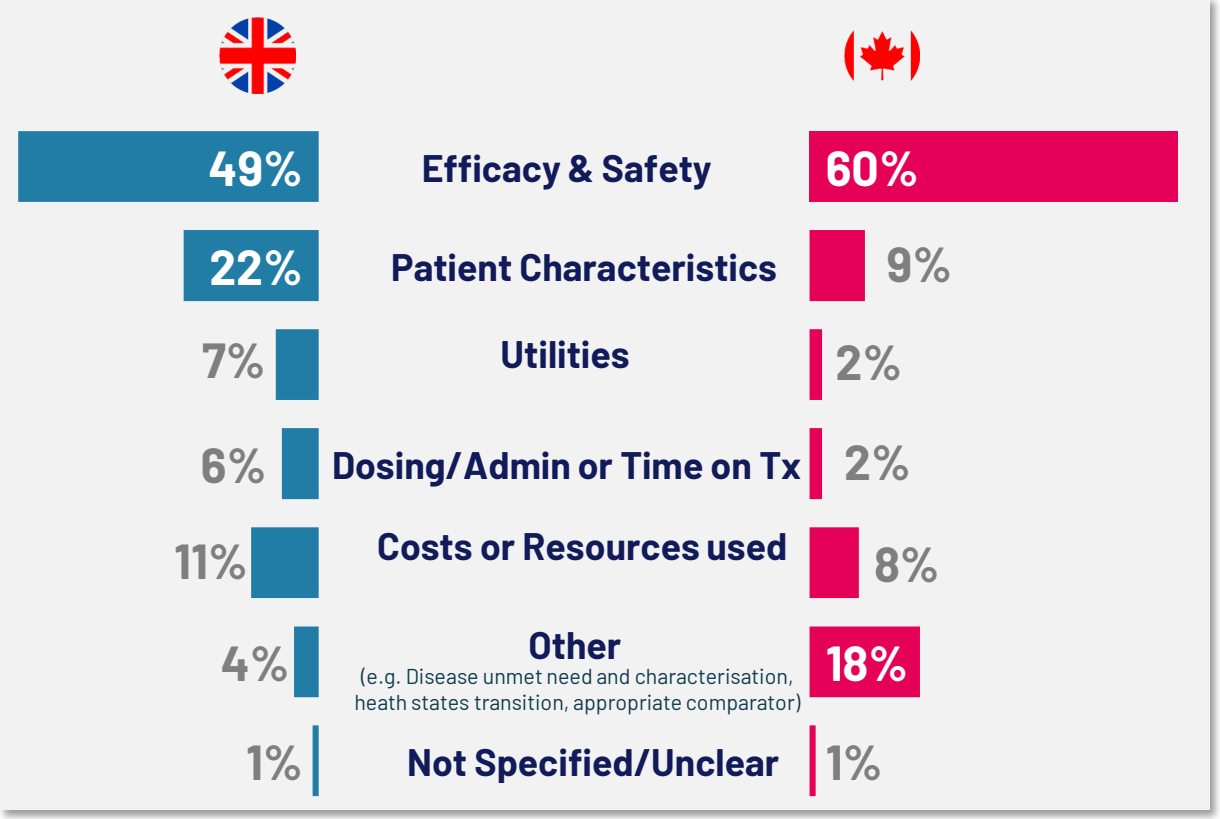


Registries and EHR are standard sources of RWE studies submitted for HTA, capturing clinical and patient characteristic data

Sources of RWE in the studies



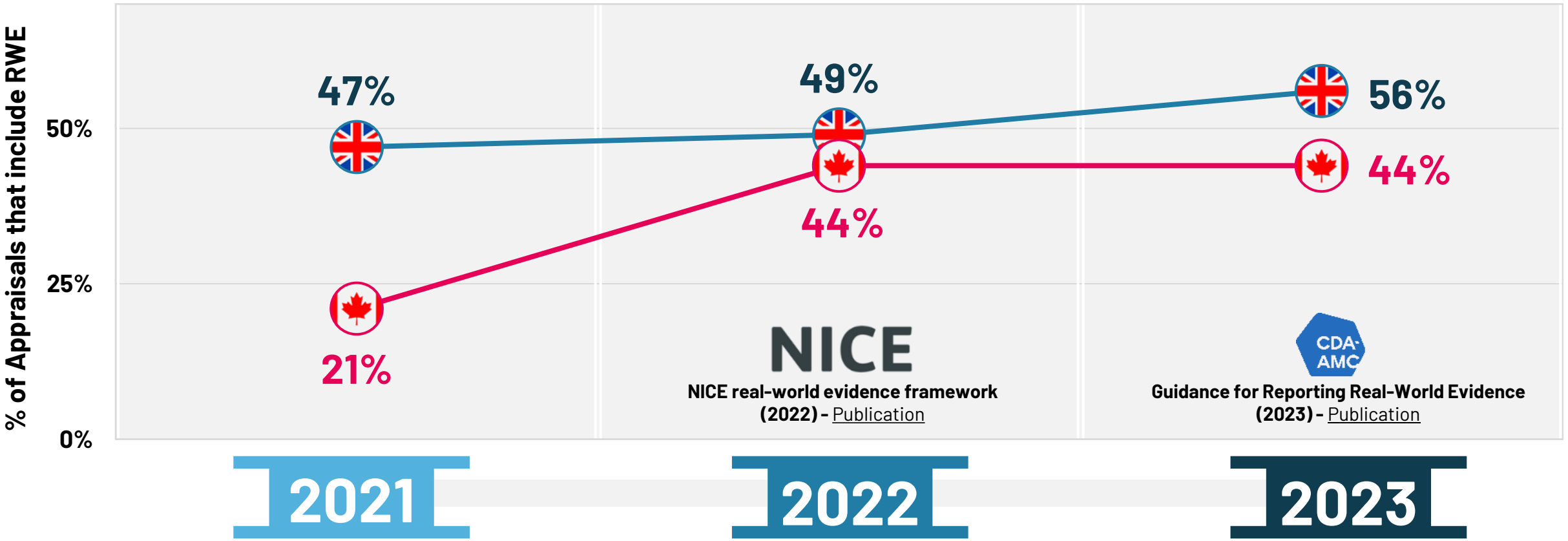
Purpose of submitted RWE studies



Patient-Generated Health Data - device-generated data on patient status
EHR – Electronic Health Records
Tx - Treatment

RWE inclusion in submissions keeps rising, possibly driven by NICE framework in UK, while CDA's guidance impact is yet to be observed

Evolution of RWE inclusion in HTA submissions



New guidance has the potential to reshape RWE submission landscape; the impact of its implementation must be monitored in the coming years

Why are most HTA appraisals, that include RWE, in the oncology space?

High costs, validated outcomes, and frequent outcome-based agreements likely contributed to the early set up of registries & EHR for oncology, now routinely used for HTA.

What characterises RWE studies submitted to HTA in the last 3 years?

Registries and EHR are the most used sources of RWE studies and are likely to remain so. **Still, the use of patient-generated health data in UK and administrative claims, for example, indicates that not all HTA-relevant RWE is in common sources and denotes innovative alternatives.**
There are less economic/costs RWE submitted in HTA likely due to its country-specific nature.

How can CDA and NICE guidance impact RWE submissions' momentum?

They enhance evaluation transparency and encourage RWE submissions. As 'living' documents, further alignment is acknowledged, and their framework doesn't appear restrictive, as evidenced by rising HTA submissions with RWE in the UK.
There is still potential in guidance to establish acceptance thresholds and ensure transparency in how RWE informs decision-making.

Patient-Generated Health Data - device-generated data on patient status
EHR – Electronic Health Records

THANK YOU

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