Integrating Real-World Evidence into Oncology Health Technology Assessment Submissions: Recent EU Examples

HTA209

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Background

- RWE in oncology health technology assessments (HTAs) is increasing in Europe to address limitations in traditional clinical trial data, particularly in single-arm studies, rare cancers, or accelerated approvals.
- The Joint Clinical Assessment (JCA) was implemented in the EU starting January 12, 2025, aiming to streamline health technology assessments for new cancer medicines and medical devices (as of 30 September 2025 nine applications are under review).

Objectives

- **The aim of this research was to review RWE included in oncology HTA submissions in selected European countries and to identify barriers to its successful application.**

Methods

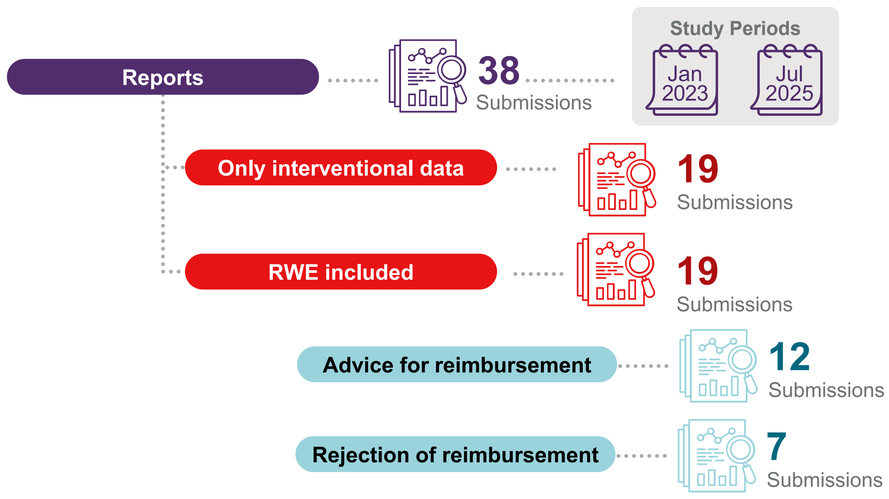
- A targeted review of oncology HTA submissions from January 2023 to July 2025 in three European countries (Netherlands, Sweden, and United Kingdom) was conducted.
- None of the included HTA submissions fall under the new JCA framework (i.e., not new cancer medicines).
- Relevant data was extracted from the submissions in the reports, including reimbursement recommendation decisions, type of RWE studies, and reasons for rejection. This data was summarized into a standardized data extraction form.
- Standard of care (SoC) data is defined here as RW data that either stem from prospective or retrospective studies and was not clearly defined in the assessment reports.
- The data are summarized with descriptive statistics, by presenting the number and proportion of advice reports in different categories.

Results

The Netherlands

- Of the 38 submissions, 19 (50.0%) included RWE, and 19 (50.0%) included only randomized-controlled trial (RCT) data.
- Twelve out of 19 submissions (63.2%) were given advice to be reimbursed (**Figure 1**).
- Nine submissions (47.4%) included data from retrospective studies, six (31.6%) used real-world data as external control arms, three (15.8%) used data from prospective studies, and one did not describe the type of data.
- Of the seven submissions for which reimbursement was rejected, "*lack of a direct comparison*" was the most common reason for rejection, followed by *"issues with patients' representativeness."*

Figure 1. Zorginstituut Nederland Submissions

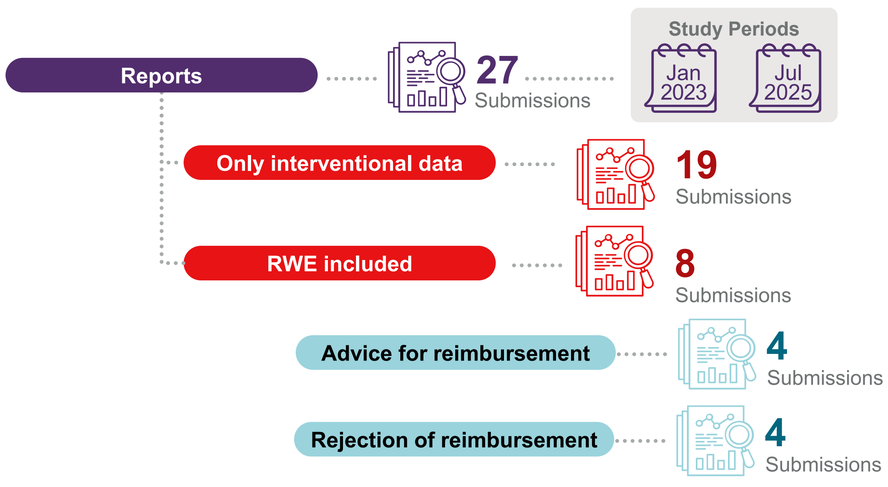


RWE = real-world evidence; ZiN = Zorginstituut Nederland (National Health Care Institute)

Sweden

- Of the 27 submissions, eight (29.6%) included RWE, and 19 (70.4%) included only RCT data.
- Fifty percent of the submissions (4/8) including RWE received advice for reimbursement (**Figure 2**).
- Three submissions (37.5%) included data from prospective studies, two (25.0%) included SoC data, and the remaining three included either retrospective data or data from a registry/database.
- Of the four submissions for which reimbursement was rejected, "*lack of a direct comparison*" was the most common reason for rejection, followed by "*uncertainty in the analysis/methodology.*"

Figure 2. TLV Sweden Submissions



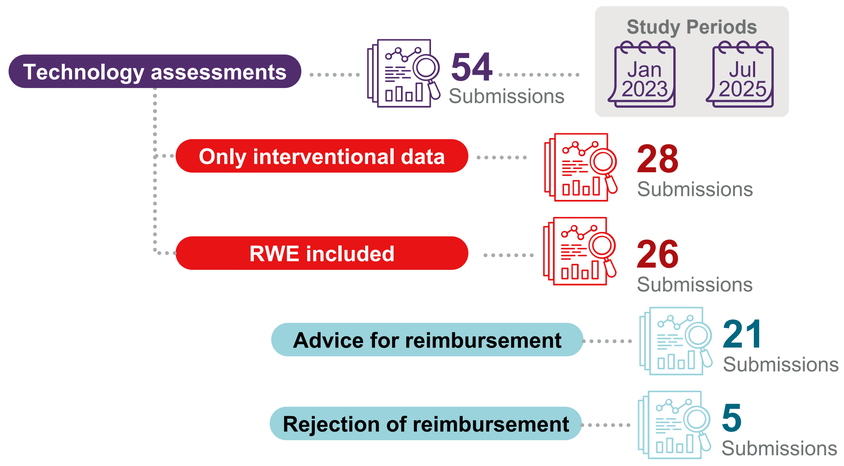
Abbreviations: RWE = real-world evidence; TLV = Tandvårds- och läkemedelsförmånsverket (Dental and Pharmaceutical Benefits Agency)

Results (cont.)

United Kingdom

- Of the 54 submissions, 26 (48.1%) included RWE, and 28 (51.9%) included only RCT data.
- Twenty-one of the 26 (80.8%) submissions that included RWE were approved for reimbursement (**Figure 3**).
- Nine (34.6%) submissions included SoC data, five (19.2%) used retrospective data, five included meta-analyses, and two did not describe the type of data.
- Of the five submissions for which reimbursement was rejected, "*immaturity of data*" and "*heterogeneity of populations*" were reasons stated for rejection.

Figure 3. National Institute for Health and Care Excellence Submissions

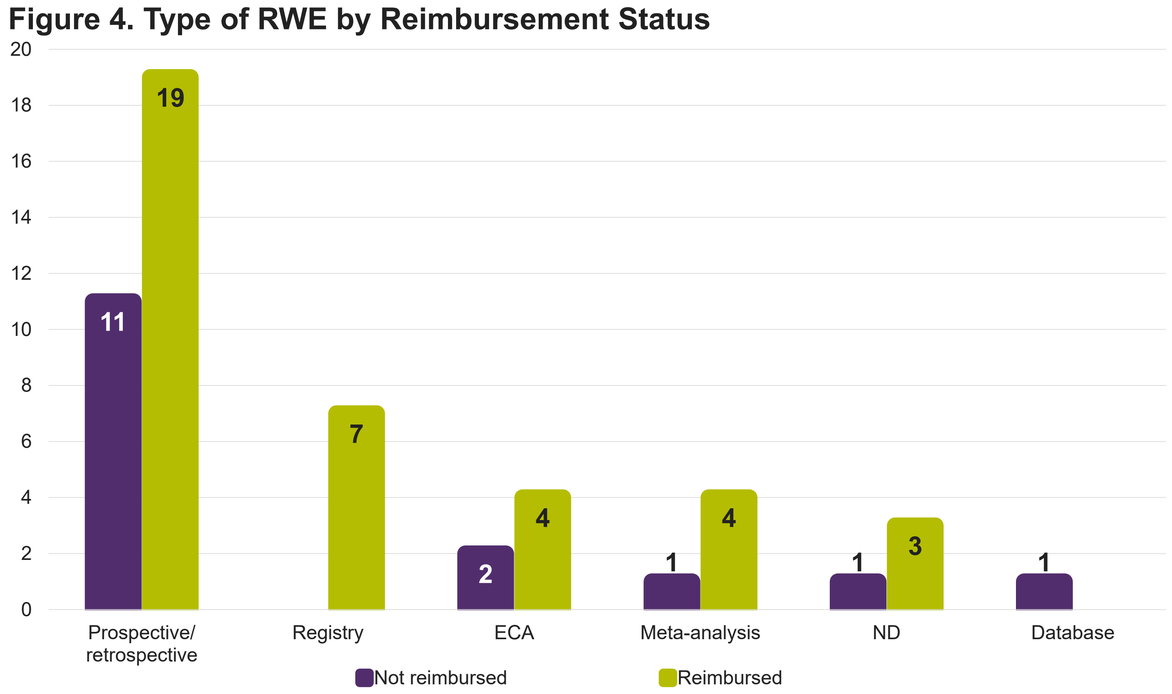


Abbreviation: RWE = real-world evidence

Summary of Submissions from All Countries

- RWE supplemented RCT data but was not used as primary evidence in any HTA submissions.
- Irrespective of reimbursement status, retrospective and prospective data (n=30) were the most frequent types of RWE included in HTA submissions, followed by registry data (n=7) (**Figure 4**).

Figure 4. Type of RWE by Reimbursement Status



Abbreviations: ECA = external control arm; ND = not determined; RWE = real-world evidence

Conclusions

- **Presence of RWE differed across countries in oncology drugs decision for reimbursement.**
- **RW study designs were diverse and RWE were complementary to trial data rather than used as standalone evidence.**
- **Submissions with supplementary RWE were accepted when studies employed robust methodologies and when RW data incorporated was used as direct comparators.**
- **Extent of RWE in HTA submissions might grow if rigorous standards are applied.**
- **With the newly implemented JCA framework, a more consistent use of RWE in submissions are expected (except for UK).**

Disclosures

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