Acceptability of Real-World Evidence by European HTA bodies to Support Value Assessments of Rare Disease Therapies

HTA360

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BACKGROUND & OBJECTIVES

BACKGROUND

- Real-world evidence (RWE) can be used alongside data from clinical trials to inform health technology assessment (HTA) of pharmacological interventions
- For rare disease therapies, collecting robust clinical evidence can be challenging due to low patient numbers, limited awareness of the disease's natural history, and lack of randomised controlled trials as a result of ethical or feasibility constraints
- RWE is particularly useful to support value assessments of non-oncology orphan medicinal products (non-onco OMPs)
- Even with the availability of published guidance in Europe, there remains a lack of stakeholder knowledge around the use and acceptance of RWE in HTAs for non-onco OMPs

OBJECTIVES

- The objective of this study was to understand the extent to which RWE is included as part of value assessments of non-onco OMPs across four European HTA bodies, and the proportion of reports with RWE that receive a positive recommendation

METHODS

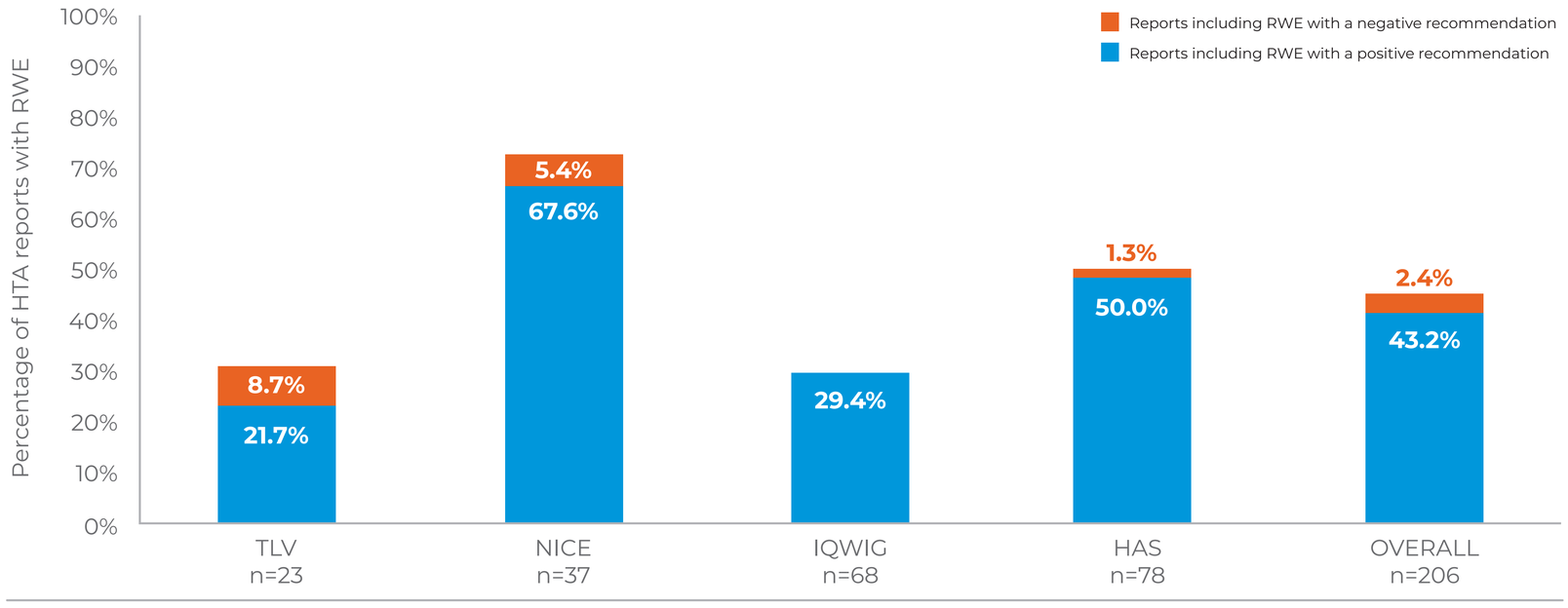
- The European Medicines Agency (EMA) database was reviewed to identify non-onco OMPs approved within the last 5 years (2018–2023) that included RWE as part of their submission package, under the assumption that the inclusion of RWE in HTA submissions may align with the inclusion of RWE in EMA-approved regulatory submissions
- Corresponding HTA reports across four European HTA bodies (National Institute for Health and Care Excellence [NICE], England; Haute Autorité de Santé [HAS], France; Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen [IQWiG], Germany; Tandvårds och läkemedelsförmånsverket [TLV], Sweden) were analysed to establish whether RWE was considered in their recommendations, and how this differed between regions
- Additionally, all HTA reports identified were examined to establish:
  - The real-world study designs used in identified reports (e.g. retrospective observational studies, registry studies, patient-reported outcome studies)
  - The sections of HTA submissions supported by RWE (i.e. clinical, safety, quality-of-life [QoL], economic)
  - The therapeutic areas in which RWE was evaluated

RESULTS

Inclusion of RWE in HTA Submissions and Resultant Recommendations

- Approvals for 105 non-onco OMPs were identified in the EMA database, for which 206 HTA reports were available (TLV: n=23; NICE: n=37; IQWiG: n=68; HAS: n=78)
- Of the 206 HTA reports, 94 (45.6%) included RWE (**Figure 1**)
- Of the 94 reports that included RWE, 94.7% (n=89) had positive recommendations (Figure 1); the breakdown by HTA body was 71.4% (n=5) for TLV, 92.6% (n=25) for NICE, 100% (n=20) for IQWiG, and 97.5% (n=39) for HAS

Figure 1: Of the non-onco OMP HTA reports identified, most of the reports that included RWE received positive recommendations

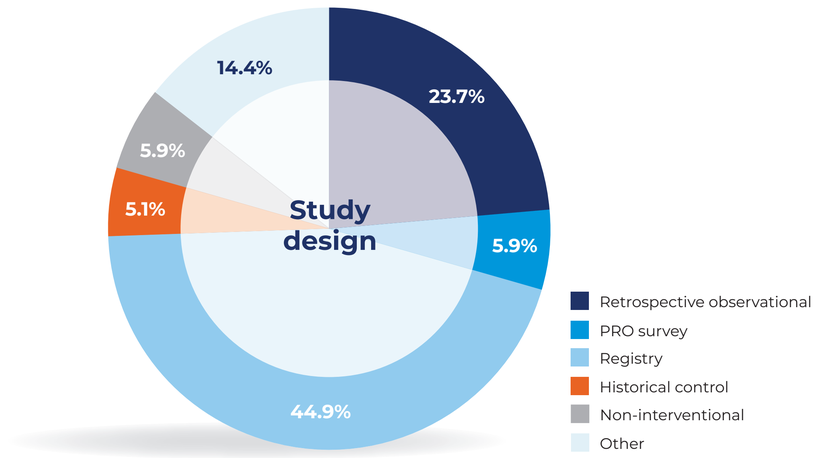


	TLV n=23	NICE n=37	IQWIG n=68	HAS n=78	OVERALL n=206
Number of HTA reports which included RWE	n=7/23	n=27/37	n=20/68	n=40/78	n=94/206
Number of reports including RWE with a positive recommendation	n=5/7	n=25/27	n=20/20	n=39/40	n=89/94

Abbreviations: HAS, Haute Autorité de Santé; HTA, health technology assessment; IQWiG, Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen; NICE, National Institute for Health and Care Excellence; non-onco OMP, non-oncology orphan medicinal product; RWE, real-world evidence; TLV, Tandvårds och läkemedelsförmånsverket.

RWE Study Design

Figure 2: Registry studies were most frequently used to gather the RWE included in non-onco OMP HTA reports

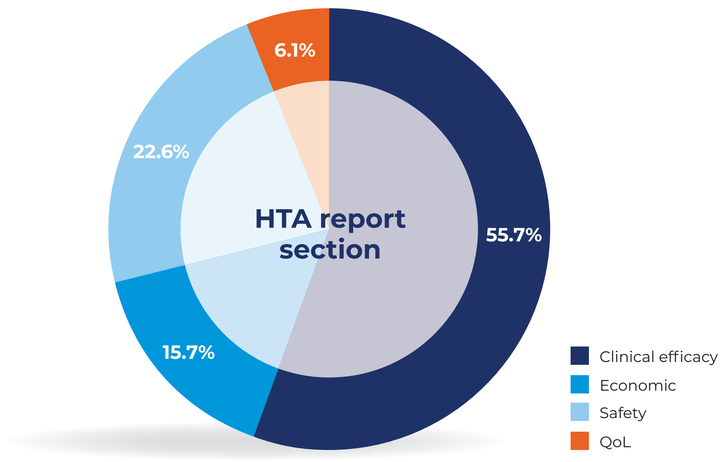


Each HTA report may include multiple types of RWE study design. 'Other' types of RWE study design included retrospective cohort, prospective cohort, healthcare practitioner survey, natural history study, electronic medical record, prospective observational, systematic literature review/network meta-analysis, administrative database analysis, patient preference, and indirect treatment comparison using RWD.

Abbreviations: HTA, health technology assessment; non-onco OMP, non-oncology orphan medicinal product; PRO, patient-reported outcome; RWD, real-world data; RWE, real-world evidence.

HTA Submission Sections Supported by RWE

Figure 3: RWE was most frequently used to support the clinical efficacy of non-onco OMP HTA reports

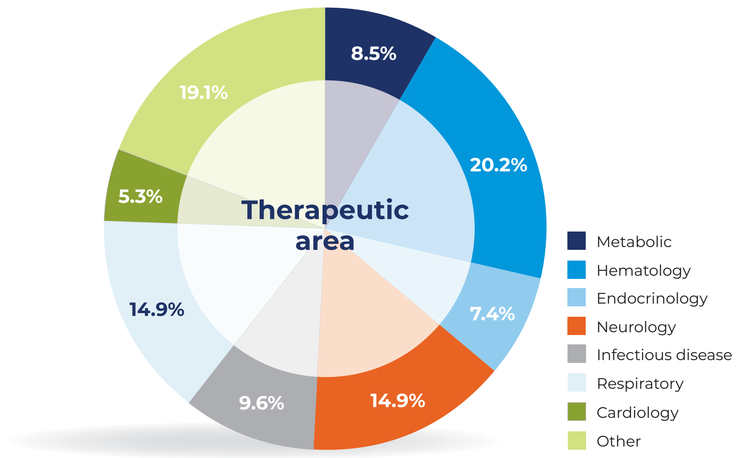


Each HTA report may include RWE which supports multiple sections.

Abbreviations: HTA, health technology assessment; non-onco OMP, non-oncology orphan medicinal product; QoL, quality-of-life; RWE, real-world evidence.

Therapeutic Areas

Figure 4: RWE was included in non-onco OMP HTA reports across a wide variety of therapeutic areas



'Other' therapeutic areas supported by RWE included immunology, rheumatology, hepatic, dermatology, nephrology, gastrointestinal, and multiple therapeutic areas.

Abbreviations: HTA, health technology assessment; non-onco OMP, non-oncology orphan medicinal product; RWE, real-world evidence.

Limitations

- This study offers insight into the use of RWE in submissions to HTA bodies in Europe; however, it was not possible to establish whether RWE was a determinant factor in final HTA recommendations, therefore further qualitive research is required

CONCLUSION

- RWE was included in a considerable proportion of submissions for non-onco OMPs to European HTA bodies, across a wide variety of therapeutic areas
- Most of the reports that included RWE received positive recommendations
- RWE was most frequently used to support the clinical efficacy section of HTA submissions; across geographies, registry studies were the most common source of submitted RWE

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