Payer perspective on the impact of real-world evidence in health technology assessments of precision oncology treatments across Europe: Results from an online survey

ISPOR EU 2024
HTA264

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Introduction

- New precision oncology therapies offer patients with specific, actionable genetic biomarkers or mutations targeted therapies that may improve survival outcomes.
- Clinical assessments and health technology assessments (HTA) of precision therapies may prove challenging due to limited sample size and expedited trials often leading to single-arm or open-label trials.
- Real-world evidence (RWE) have been presented as a potential approach to supplement evidence packages for regulatory and HTA assessments; however, European HTA bodies have not been aligned on the utility and acceptance of RWE in reimbursement decisions.

Objectives

- **Explore use of external control arms (ECAs) derived from RWE in initial assessments of precision oncology therapies**
- **Explore the role of RWE in HTA/payer reassessment of oncology drugs in the post-launch period**
- **Assess HTA/payer perception on valuable real world (RW) data qualities and RWE study designs and methodologies**
- **Assess importance of having local RWE vs acceptance of data from another country**

Methods

- A web-based survey administered via the Rapid Payer Response (RPR™) platform by Genesis Research Group was administered to 25 payers with HTA and reimbursement decision making responsibilities for precision oncology therapies across Europe.
  - Payer profiles included ex-NICE (UK), ex-CEPS and ex-TC (France), ex-G-BA and SHI (Germany); ex-national and regional payers (Italy and Spain).
- Respondents were asked on their perceptions of RWE specifically in the reimbursement of precision oncology therapies

Limitations

- Results represent the opinions of a select group (N=5) of payers and reimbursement decision makers from each country. Larger sample of respondents may use different results.
- The context of the survey was specific to precision medicine in oncology; use and acceptance of RWE in other therapeutic areas may differ than what is reported here.

Conclusions

- **Comparability, completeness and generalizability of data are key factors when assessing a RW data source of use with HTAs.**
- **Preference for regional data may pose a substantial hurdle to industry when suggesting RWE approaches; although payers recognize the limitations of regional data and prioritize fit-for-purpose data.**
- **Industry should continue to work with HTA bodies to understand the optimal design and execution of RWE projects for maximized likelihood of HTA acceptance.**

Results

- The use of RWE in ECAs to support regulatory submission, pricing, and reimbursement varied by country surveyed (Table 1).
  - UK and Italy showed a more favorable perception of the potential impact of RWE in pricing and reimbursement and had high levels of consensus amongst the payers surveyed.
  - The opinions of German payers varied; however, the overall perception was that RWE was either not considered at all or provided context without significant impact.
- Common exceptions to the payer perceptions included cases where no head-to-head comparator is feasible, rare diseases with small patient populations, and cases with high unmet need.

Results

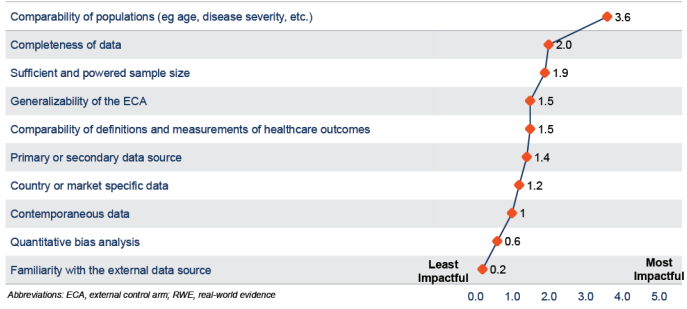
Table 1. Perceptions of Impact of RWE in ECAs for Novel Therapy Pricing and Reimbursement

Payer Market	Perception of Impact of RWE in Pricing and Reimbursement	Exceptions (Cases of…)
	Either not considered at all or considered as context with no significant impact on pricing or reimbursement	• dramatic effect • no head-to-head comparator is feasible
	Can support case for reimbursement but likely no impact on pricing	• control group is poorly chosen • no head-to-head comparator is feasible • rare disease / small patient populations • high unmet need in the population
	Can support case for reimbursement but likely no impact on pricing	• rare disease / small patient populations • Standard of care (SoC) is different in Spain than trial
	Can support both pricing and reimbursement	• rare disease / small patient populations
	Can support both pricing and reimbursement	• rare disease / small patient populations • high unmet need in the population • SoC is different in the UK than trial

Abbreviations: ECA, external control arm; RWE, real-world evidence; SOC, standard of care; UK, United Kingdom

- When asked to rank the top 5 data attributes amongst a list of 10, the 5 most important were comparability of populations in RW to clinical trial, completeness of RW data, sample size, generalizability of ECA data, and comparability of outcomes in RW to clinical trial (Figure 1).
- Results varied by region and individual payer.

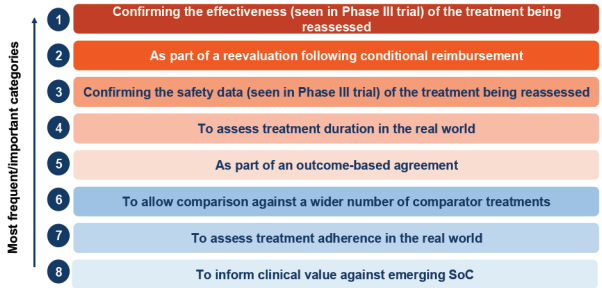
Figure 1. Data source qualities most impactful for RWE comparator arms



Abbreviations: ECA, external control arm; RWE, real-world evidence

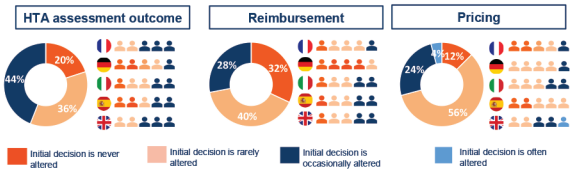
- For reassessments, RWE is most used to confirm the effectiveness and safety of the treatment under assessment (Figure 2).
  - On occasion, RWD can be used to confirm assumptions around treatment duration used in cost-effectiveness or budget impact models.
  - While RWE could be leveraged during implementation of any outcome-based agreements, low appetite for such contract structures reduces this use case.

Figure 2. Payer ranking use of RWE in reassessments



Abbreviations: RWE, real-world evidence; SoC, Standard of care

Figure 3. Impact of RWE in reassessments

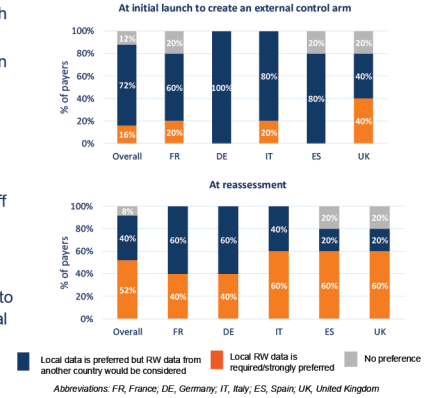


Abbreviations: HTA, Health Technology Assessment

- Responses suggest that while RWE can occasionally result in a changed HTA assessment outcome at reevaluation, mainly in France and the UK, it rarely has an impact on the reimbursement or pricing of a product (Figure 3).

- Most payers across scope markets prefer local-level data (Figure 4).
  - Still a majority recognize limitations in availability of local data at product launch, accepting RWE from other European markets with similar demographics and comparable healthcare systems
  - At reassessment, preference for local data increases, especially in situations where a mandatory data collection requirement was agreed at launch (e.g., registry requirement, CDF coverage)
  - Respondents emphasized that in situations where local data of sufficient quality are not available, data from similar healthcare systems or populations can be leveraged.
- Despite the general preference for local data, when asked to trade off data origin vs appropriateness, most stakeholders prioritize fit for purpose data over country of origin.
- A minority of respondents maintained their preference for local data:
  - The National Health Services (NHS) can leverage assumptions to fill data gaps with higher confidence if data is gathered from local patients – (1×UK)
  - Data from another country can supplement local data, but inclusion of local data is a must (1×FR)

Figure 4. Payer Acceptance of Local vs Non-Local Data



Abbreviations: FR, France; DE, Germany; IT, Italy; ES, Spain; UK, United Kingdom

Disclosures. This study was conducted by Genesis Research Group and funded by Genesis Research Group. All authors are employees of Genesis Research Group.

Acknowledgements. We would like to thank our payers for their enthusiastic participation in this research.

Presented at the ISPOR EU 2024
Barcelona, Spain
November 19, 2024

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GENESIS RESEARCH GROUP

A Real-World Data Landscape Review of the 2023 International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Europe Conference

RWD128

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Introduction

- Research presented at International Society for Pharmacoeconomics and Outcomes Research (ISPOR) Europe conferences and published in the society's journal, *Value in Health*, offer insight into real-world data (RWD) use in the life sciences industry.
- A large proportion of abstracts submitted to the ISPOR family of conferences utilize a variety of RWD sources to generate real-world evidence.
- Regulators continue to sharpen guidance on real-world evidence, including fit-for-purpose data selection; therefore, it is important to describe the current real-world data landscape.

Objective

To quantify and characterize RWD sources utilized for research described in 2023 ISPOR Europe abstracts

Methods

Data Source

- The *Value in Health* December 2023 supplemental issue, which includes all ISPOR Europe 2023 conference abstracts, served as the source of research abstracts.

Research Abstract Sample

- To identify the subset of abstracts describing RWD utilization, abstracts were filtered based on a case insensitive text search of their 'Methods' sections.
- Abstracts were included if methods described direct analysis of RWD (i.e., excluding literature reviews) and identified a RWD source either by name or RWD source category.

Search Terms

"database", "data base", "real world", "real-world", "claims", "electronic health", "real-world data", "linked", "token", "survey", "registry", "electronic medical", "ehr", "emr"

- Abstracts that contained these search terms were included in the sample for additional manual review by two reviewers.
- Reviewers documented the RWD source characteristics from each abstract including:
  - RWD Source Name
  - RWD Source Type (e.g., administrative claims)
  - Country/Countries included (if data from >1 country was cited, all countries mentioned were included in the measure for figure 3)
  - Tokenized / Linked Data Source (e.g., claims+EHR)

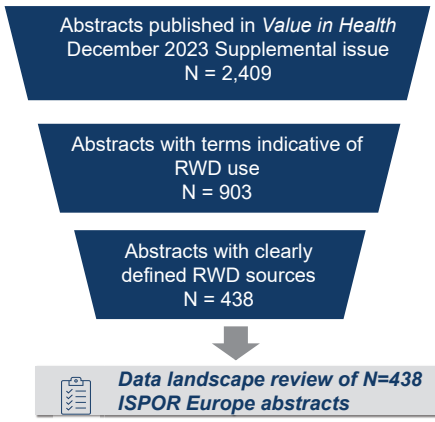
Statistical Analyses

- Abstract characteristics were summarized with descriptive statistics

Results

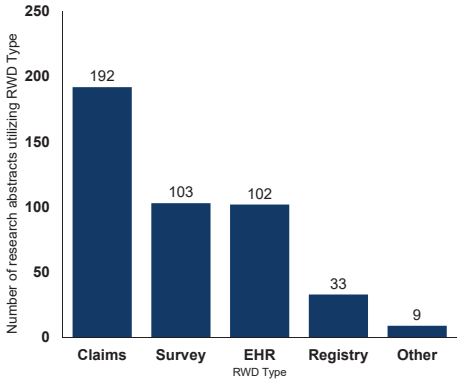
- Among the 2,409 ISPOR abstracts presented at ISPOR Europe 2023, 903 (37%) mentioned utilization of RWD, and 438 met inclusion criteria for RWD source description **Figure 1**

Figure 1. Research Abstract Selection



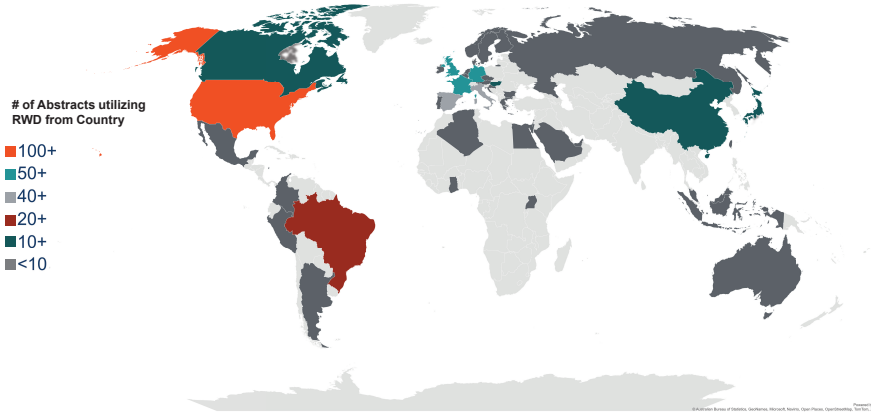
- **A total of 170 unique RWD sources** were cited in the N=438 abstracts

Figure 2. RWD Sources Utilized for Research in ISPOR Europe 2023 Abstracts by Type



- The most common RWD source type utilized for research was administrative claims (N=192 abstracts [44%]) **Figure 2**
- A total of 15 (3.4%) abstracts cited utilizing multiple RWD sources, for example: combination of administrative claims with survey data

Figure 3. Putting the "I" in ISPOR: RWD Sources by Geography



- The majority of RWD sources included data from from the U.S. (N=110 abstracts; 25.1%)
- Half of the abstracts utilized data from at least one European country (N=219 abstracts; 50%);
- Many abstracts used RWD from EU5 countries: France (n=74), United Kingdom (n=60); Germany (n=59), Italy (n=41), Spain (n=40) **Figure 3**

Limitations

- Our search terms may have omitted non-standard RWD sources from the manually reviewed abstract sample.
- Descriptions of the RWD sources and/or specific data source names may have been abbreviated due to abstract word limits.

Conclusions

- **The current RWD data landscape is well-developed with hundreds of sources, each having unique strengths and characteristics.**
- **Administrative claims remain the most utilized source; however, claims alone may not be fit for all research questions.**
- **A data-agnostic strategy and updated knowledge of the global data landscape are beneficial for selecting fit-for-purpose RWD.**

Disclosures

This study was conducted by Genesis Research Group and AS is an employee of Genesis Research Group. ET was an employee of Genesis Research Group when the research was completed.

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Poster presented at ISPOR Europe 2024 - Barcelona, Spain, November 17-20, 2024