# EVOLVING PAYER PERCEPTION ON PATIENT CENTRICITY AND REAL-WORLD EVIDENCE: INSIGHTS FROM A QUANTITATIVE SURVEY

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## INTRODUCTION

- Regulatory bodies, including the Food and Drug Administration (FDA) and European Medicines Agency (EMA), endorse real-world evidence (RWE) and generation of patient-centric evidence as part of the patient-focused drug development process, encouraging manufacturers to conduct these studies
- This research defined RWE as clinical evidence on usage and potential benefits or risks of a medical product derived from an analysis of real-world data and patience-centric evidence as research that captures patients' experiences/perspectives through randomized clinical trials (RCTs) and/or RWE

## OBJECTIVES

- To understand payer receptivity to and perception of RWE and patient-centric research conducted as a part of clinical trials or RWE, as well as the impact of such evidence on payer decision-making
- To captures trends across USA and EU payer organizations(s) in accepting evidence generated using real-world studies with diverse methodologies and data sources for drug evaluations / reimbursement decisions

## METHODS

- A quantitative survey was conducted with 18 payers from France (FR), Germany (DEU), Spain (ESP), the United Kingdom (GBR), and the United States (USA) (Table 1)
- Data were collected via a web-enabled instrument, including a screener to ensure relevant payers were part of the research, followed by a 15-minute questionnaire
- Payers were selected from Trinity Life Science's payer expert repository; payers with relevant knowledge about RWE and patient-centric research were prioritized for participation in this study

**Table 1** | Payer Characteristics

| Geography | Total N | Type of Payer Organization, N (%) | Years of Experience (Mean) |
|---|---|---|---|
| FRA | 3 | N=2 (67%) TC; N=1 (33%) CEPS | 15.0 |
| DEU | 4 | N=3 (75%) G-KV; N=1 (25%) G-BA | 17.8 |
| ESP | 4 | N=4 (100%) Regional | 16.8 |
| GBR | 3 | N=1 (33%) NICE; N=2 (67%) CCG | 18.3 |
| USA | 4 | N=3 (75%) National / Regional MCO | 17.8 |

Abbreviations: CCG: Clinical Commissioning Group; CEPS: Comité Economique des Produits de Santé; G-BA: Gemeinsamer Bundesausschuss; G-kV: Gesetzliche Krankenversicherung; MCO: Managed Care Organization; NICE: National Institute for Health and Care Excellence; TC: Transparency Committee

## RESULTS

### Acceptance of RWE in Drug Evaluations

- EU payers (100% in FRA, 75% in DEU) started accepting RWE in the late 1990s / early 2000s, whereas 50% of USA payers started accepting it in the late 2000s
- 25% of USA payers and EU payers (75% in ESP) indicated RWE may be increasingly accepted in the coming years

**Figure 1** | **Influence of RWE and Patient-Centric Evidence in Payer Decision-Making**

**Key Findings:**
- RWE and patience-centric evidence currently has limited influence on payers as it is viewed as complementary to clinical trial evidence
- RWE and patient-centric evidence was indicated as being impactful in rare or severe disease conditions and relevant for identifying comparator therapies

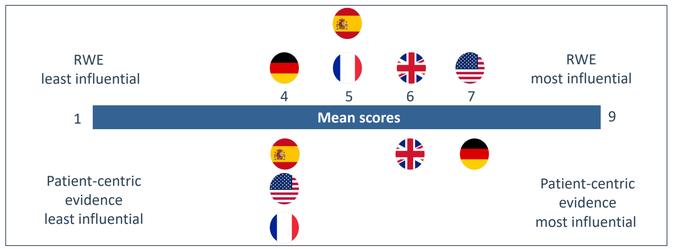



**Figure 2** | **Payer Perception of Most Impactful Types of RWE in Drug Evaluations**

**Key Findings:**
- The majority of payers stated that secondary data from network meta-analyses (NMAs) were the most impactful type of RWE; DEU payers indicated that patient surveys conducted using validated patient-reported outcome (PRO) measures were the most important for them
- Payer perceptions varied on long-term registry studies, secondary data analysis using claims database, and primary data research using medical charts

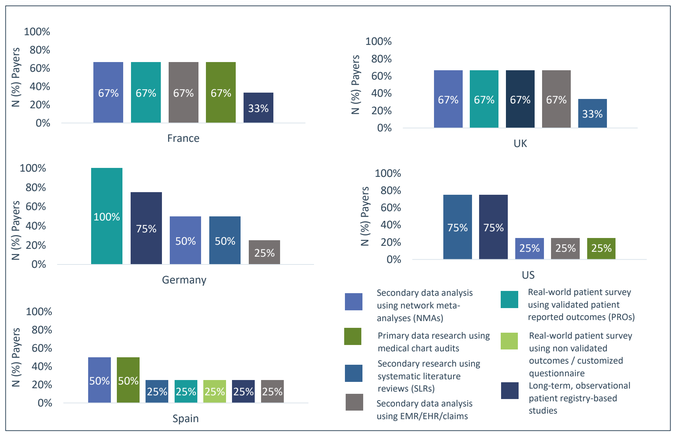



**Table 2** | **Payer Description of Patient-Centric Research***

| Country[1] | UMN | SB | QoL and ADL | Pref TS | Pers TI | WP | CG burden |
|---|---|---|---|---|---|---|---|
| FRA | 67% payers | 67% payers | 67% payers | 33% payers | 67% payers | NS | 67% payers |
| DEU | 25% payers | 100% payers | 100% payers | NS | 25% payers | 25% payers | NS |
| ESP | 75% payers | 50% payers | 25% payers | 25% payers | 75% payers | NS | NS |
| GBR | 33% payers | 67% payers | 67% payers | NS | 67% payers | 33% payers | 33% payers |
| USA | 50% payers | 100% payers | 50% payers | 50% payers | 25% payers | 25% payers | NS |

Note: *Payer perception of research quantifying the above options as patient-centric research; UMN: Unmet needs; SB: Symptom burden, Pref TS: Patient preference for treatment selection; Pers TI: Patient perspective on treatment improvement, WP: Work productivity, CGB: Caregiver burden, NS: Not selected

**Figure 3** | **Current Use of Patient-Centric Evidence in Payer Decision-Making**

**Key Findings:**
- Across markets, payer description of patient-centric research differed widely; when included, payers used patient-centric evidence to understand disease burden and supplement other types of evidence
- EU payers utilized patient-centric evidence to conduct health technology assessment; 25% USA payers used this evidence to guide pricing decisions vs 33% FRA, 25% ESP, and 50% DEU payers

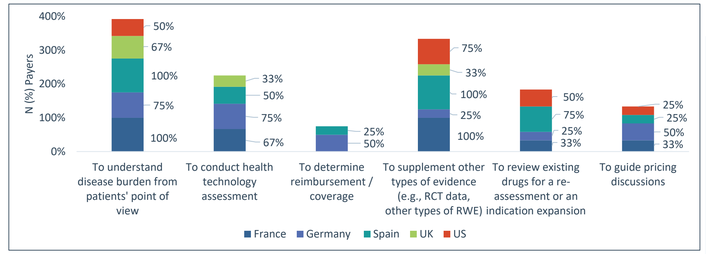



**Figure 4** | **Payer Perception of Most Valuable Patient-Centric Data**

**Key Findings**:
- Across markets, payers considered QoL, ADL, followed by disease burden as the most valuable types of patient-centric data
- Out-of-pocket costs and use of OTC medicines were seen as the least valuable; DEU, USA and GBR payers considered the need for hospitalization as a valuable patient-centric research evidence

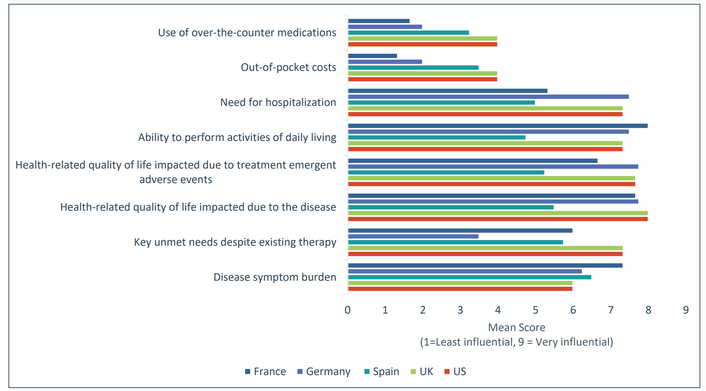
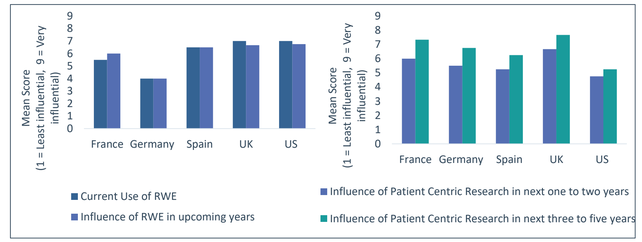



**Figure 5** | **Expected Influence of RWE and Patient-Centric Evidence in the Future**

**Key Findings:**
- Over the upcoming years, payers across markets anticipate a steady use of RWE and patient-centric evidence in HTA and reimbursement coverage decision making
- EU payers anticipate a significant increase in the influence of patient-centric evidence in payer decision-making




## DISCUSSION & CONCLUSION

### Payer Perception of RWE and Influence in Reimbursement Decisions

- Despite RWE's potential to strengthen RCT data, there exists a notable divergence in how payer organizations incorporate RWE into their decision-making, highlighting the need for further research on this topic
- Payer perspectives on the relative importance of RWE in their decision-making varied across markets, with GBR and USA payers indicating that RWE had a higher level of impact on reimbursement decisions relative to other markets
- However, all payers across USA and EU markets viewed RWE as complementary to RCT data and particularly beneficial in rare diseases where there are challenges associated with conducting an RCT

### Payer Receptivity to Different Types of RWE

- Evidence from NMAs and real-world patient surveys using validated PROs emerged as the most impactful type of RWE in most EU markets, whereas evidence from SLRs and long-term patient registry-based studies were considered most relevant in the USA

### Relative Importance of Patient-Centric Evidence in Payer Decision-Making

- Patient-centric evidence was seen as research that quantifies symptom burden and assesses QoL or ability to perform activities of daily living
- Payers generally viewed patient-centric evidence as supplemental to RCT data to understand the disease burden from patients' point of view
- As such, the level of influence of patient-centric evidence in payer decision-making was perceived to be moderate in most markets and relatively high in the GBR and DEU

### Best Practices in Patient-Centric Research & Future Trends in Use of RWE

- The use of patient-centric research and RWE in payer decision-making is expected to increase in the next few years
- However, payers recommend conducting RWE using robust study designs and credible data sources to increase the likelihood of acceptance and heighten the level of impact the evidence will have on HTA / reimbursement decisions

**Ask A Question:** akulkarni@trinitylifesciences.com

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**Disclosures:** All the authors are employees of Trinity Life Sciences (Waltham, MA), and NH and JP hold equity in Trinity Life Sciences.