# Payer perceptions of supportive evidence in the estimation of long-term durability for gene therapies in major European markets

HTA124

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## Introduction

Long-term durability of effect is the most prominent uncertainty that exists during payer assessments of gene therapies*. This is because the length of gene therapy clinical trials is typically lower than the duration of expected benefit at the time of health technology assessment (HTA)

Supportive evidence (evidence other than clinical trial data) is used increasingly to reassure payers on gene therapy durability, but the relative impact of such evidence on European HTA outcomes is uncertain

## Objective

This research aims to understand the extent to which payers in major European markets are likely to accept and value supportive evidence types in the estimation of gene therapy durability

## Methods

**Interviews held with former payers (*n*=3 per country) from:**

- **England** (ex-NICE)
- **Italy** (ex-AIFA/regional payers)
- **France** (ex-TC/CEPS)
- **Spain** (ex-AEMPS/regional payers)
- **Switzerland** (ex-FOPH)
- **Germany** (ex-GB-A/GKV-SV)

**When prompted, respondents commented & scored on acceptability and importance of randomized controlled trial (RCT) data and the following supportive evidence types for evaluating durability:**

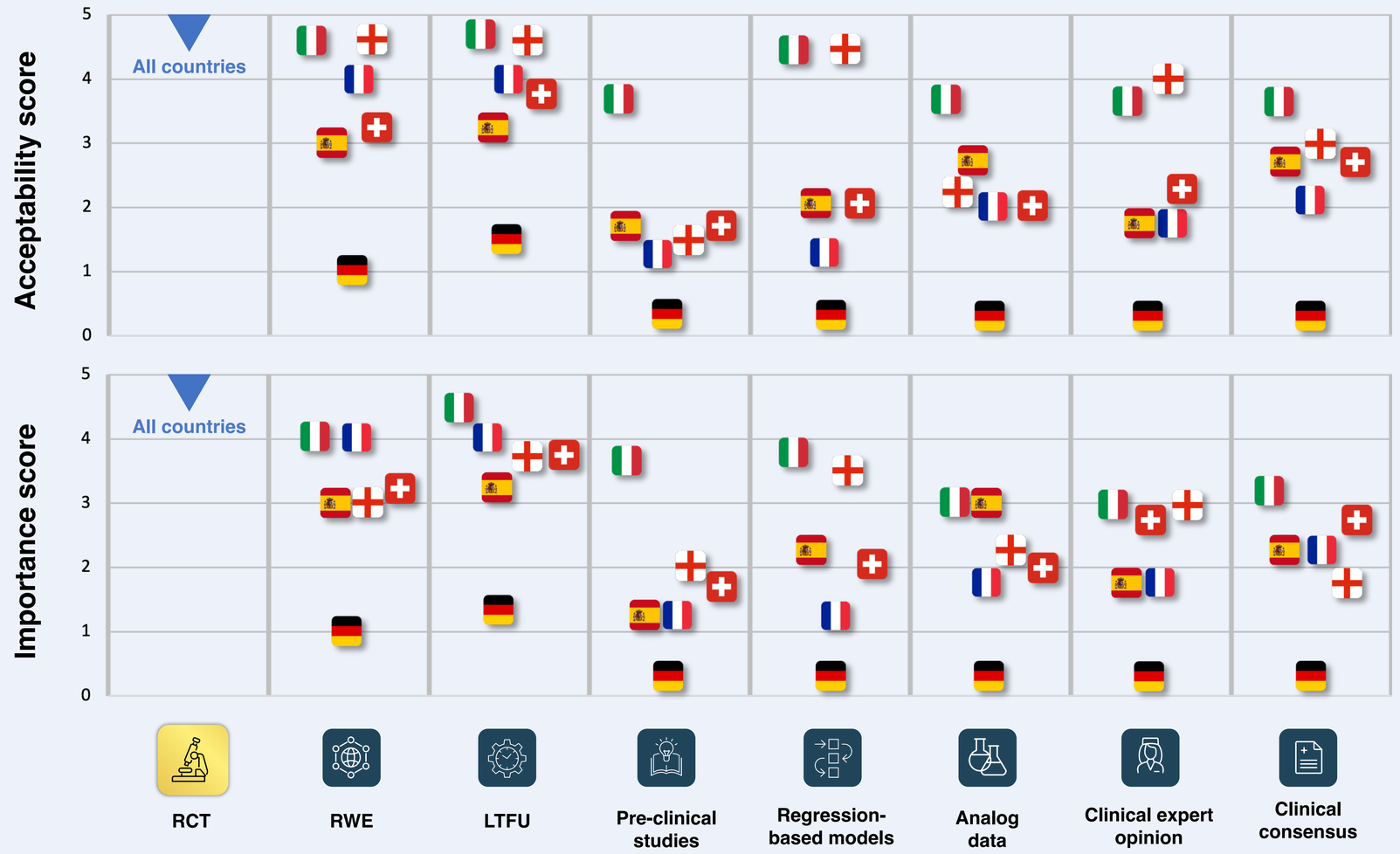
- Real-world evidence (RWE)
- Non-comparative long-term follow up (LTFU)
- Pre-clinical studies
- Regression-based modeling
- Analog data
- Clinical expert opinions
- Clinical consensus statements

## Results

**Across markets:** The concept of durability was rated as important for gene therapy decision-making, with **most payers ranking durability as important as efficacy. LTFU, RWE, regression-based modeling, analog data and clinical consensus** are likely to be the **most impactful supportive evidence types** in studied European markets (to varying degrees). **LTFU** was perceived to be the **most important supportive evidence type, followed by RWE** (if available). **Pre-clinical data** was considered the **least important supportive evidence type**

### The average ranking of evidence for payer decision-making on durability in each country

Average scores are derived from qualitative & quantitative insights (0/1 = no / very low and 5 = very high)




**Payers in Switzerland, Italy, Spain & England** showed a preference for **all available evidence types being submitted**, with justification

In **Germany and France**, supportive evidence **other than LFTU and RWE was considered of less value**

## Conclusion

**Supportive evidence for gene therapy durability is expected to be accepted and likely valued in most studied markets. RWE & LTFU carry the most value as supportive evidence for durability, which may impact HTA outcomes**

To complement clinical data in payer assessments of gene therapy durability, manufacturers should consider a broad supportive evidence package reflecting market-specific differences in the acceptability and importance of evidence types

### Study limitations

Results reflect general insights from ex-payers in a limited sample (*n*=3 per market). Comprehensive research tailored to a given gene therapy asset to shape the durability data package is recommended

## Contact

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*Source: Qiu, T. *et al.* (2022) Gene therapy evidence generation & economic analysis: Pragmatic considerations to facilitate fit-for-purpose HTA, Frontiers in public health. 10: 773629