HTA172

Treat Now, Prove Later?

EU5 Payer Perspectives on Pre-Symptomatic Gene Therapy for Huntington's Disease

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INTRODUCTION

- Huntington's disease (HD) is a fatal neurodegenerative condition caused by a single gene mutation
- Carriers can be genetically identified decades before symptoms appear, typically in their 20s–30s, with symptom onset usually not until their 40s–50s
- Emerging gene therapy has shown evidence of delaying disease progression in early-symptomatic patients; however, prescriber and patient demand to treat earlier is expected to be strong, despite clinical benefit only becoming observable 10–20+ years later

OBJECTIVE

1. To assess EU5 payer willingness to reimburse a one-time gene therapy for genetically confirmed but pre-symptomatic HD patients
2. To characterise the required evidence for reimbursement
3. To assess whether current HTA frameworks are fit for this therapeutic modality
4. To establish the preferred payment / risk-sharing agreements for the reimbursement of a one-time gene therapy for pre-symptomatic HD patients

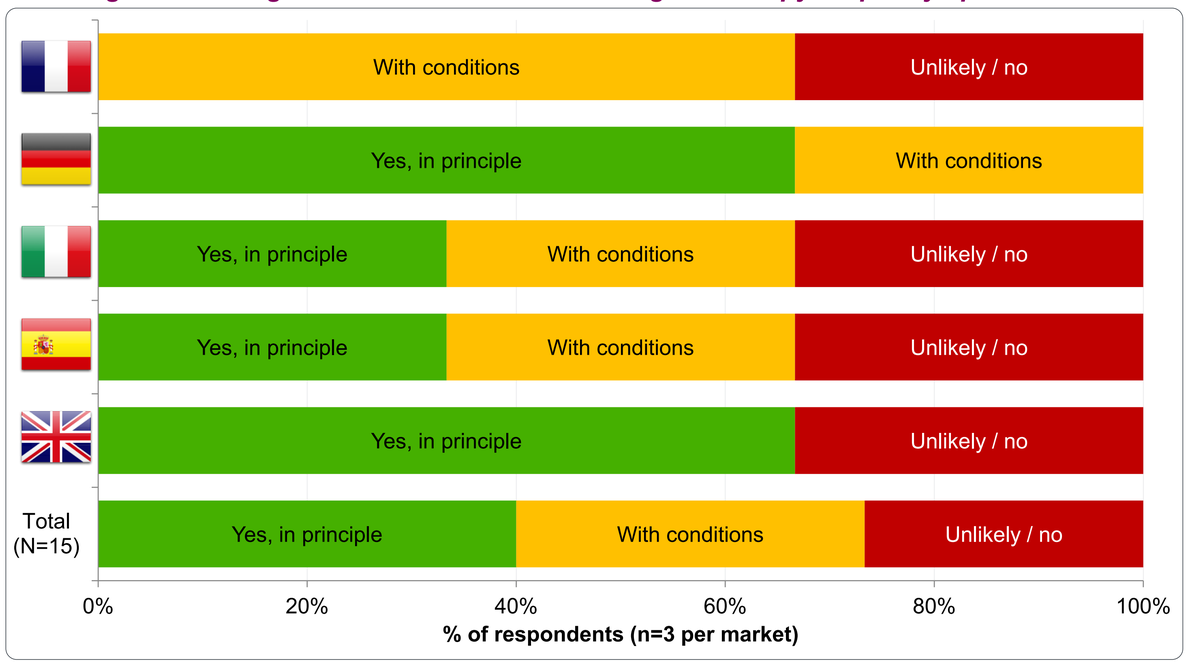
METHODS

- A structured survey was conducted with N=15 ex-payer and HTA expert respondents across the EU4+UK (n=3 per market)
- Respondents indicated, for a candidate pre-symptomatic gene therapy for HD:
  - Reimbursement willingness
  - Required evidence (surrogate biomarkers, extrapolation from early symptomatic trial data, long-term registries)
  - Appropriateness of existing HTA frameworks (including discount rates) for preventative gene therapies
  - Preferred payment / risk-sharing structures

RESULTS

Payers are open to pre-symptomatic HD gene therapy reimbursement, but only through conditional, evidence generating pathways

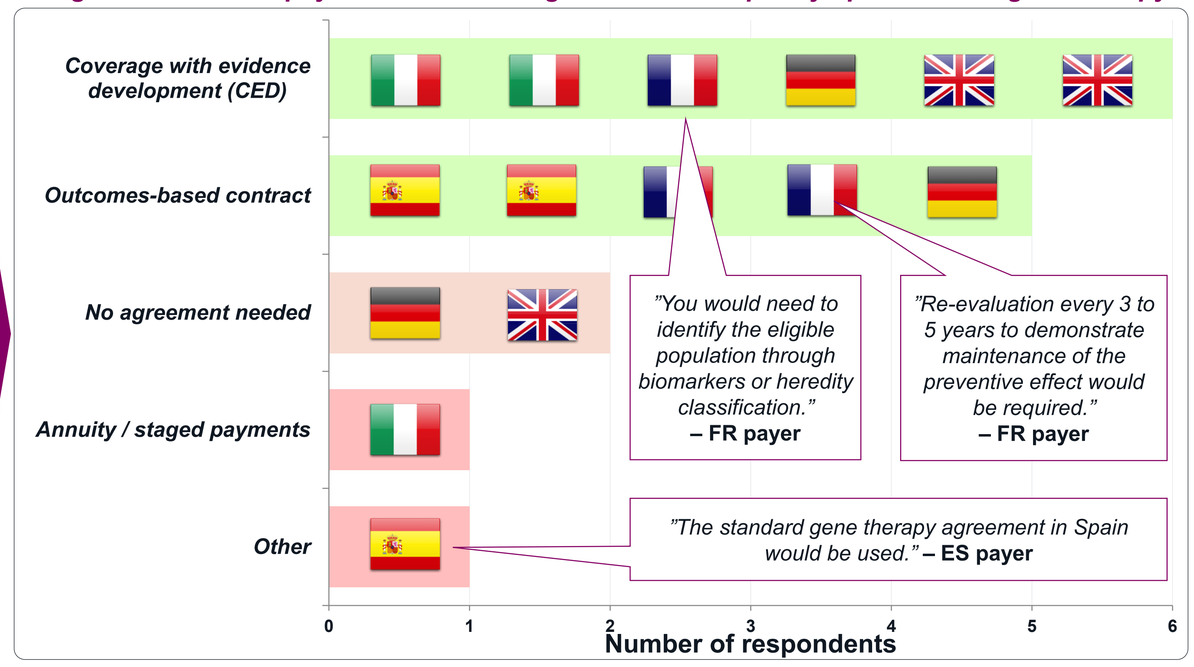
Figure 1. Willingness to reimburse a one-time gene therapy for pre-symptomatic HD



Free-text "other" responses (n=4) were coded by theme: "yes, if on label" (DE) → yes; reimbursement dependent on label evidence (DE) or pay-for-performance (FR) → conditional; preference to wait until symptomatic (ES) → unlikely/no. EU5: 6 (40%) yes; 5 (33%) conditional; 4 (27%) unlikely/no.

Payers will likely require outcomes-based agreements or evidence development for presymptomatic gene therapy reimbursement

Figure 2. Preferred payment / risk sharing for a one-time pre-symptomatic HD gene therapy



CONCLUSIONS

- Nearly **three-quarters of EU5 payers** (73%) **would consider reimbursing a one-time gene therapy for pre-symptomatic HD**
  - **Germany** and the **UK were among the most open**, while **France were the least open**
- **However, payers are unlikely to accept surrogate biomarkers or extrapolation from early-symptomatic data** on their own
  - This poses a significant challenge for manufacturers as providing **hard clinical and patient-reported outcomes** benefit data will be very difficult in the context of the timeframe of a typical clinical trial, **potentially requiring decades of patient follow-up post-treatment**
- Accordingly, innovative reimbursement approaches are broadly deemed necessary by payers, with **outcomes-based contracts and coverage with evidence generation being widely anticipated**; while **annuity payments drew little support**

REFERENCES AND ABBREVIATIONS

1. Precision AQ global pricing & market access research survey, anti-ageing therapies module, 2026: structured survey of N=15 ex-payer and HTA expert respondents across the EU5 (n=3 per market). All data presented on this poster are drawn from this primary research; respondents are identified by market only.

Abbreviations: EU5, France/Germany/Italy/Spain/UK; CED, coverage with evidence development; DE, Germany; ES, Spain; FR, France; HD, Huntington's disease; IT, Italy; UK, United Kingdom; HTA, health technology assessment; N/n, number of respondents

Disclosures: all authors are employees of Precision AQ. Presented at ISPOR Europe 2026.

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