EE208

Target Population Size and Launch Price Dynamics of Cell and Gene Therapies: Evidence from European HTA Systems

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Background

- Cell and gene therapies (CGTs) offer transformative clinical benefits for rare and high-burden diseases.[1]
- However, CGTs challenge traditional pricing frameworks due to high upfront costs and uncertain long-term outcomes.[2]
- In Europe, HTA bodies increasingly rely on value-based pricing principles, yet how population size influences pricing remains underexplored.[2,3]
- Understanding this relationship is critical for affordability, payer negotiation, and global pricing strategy.

Objectives

- Quantify the relationship between launch price and HTA-derived target population size
- Compare this relationship across Germany (G-BA), France (HAS), and England (NICE)
- Explore implications for value-based pricing and cross-market referencing

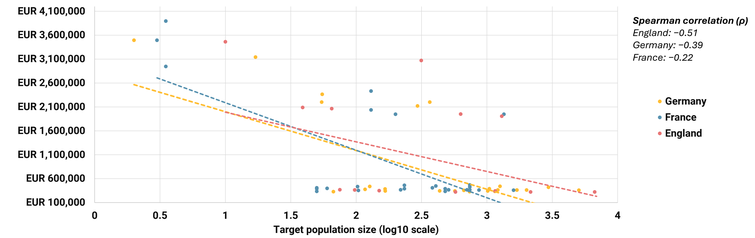
Methods

- A cross-sectional analysis was conducted using HTA-Hive database to identify HTA reports for CGTs published between 2015 and 2025.
- Estimated annual target populations were extracted from HTA reports issued by G-BA (Germany), HAS (France), and NICE (England).
- First publicly available launch prices were obtained from public sources and standardised to 2025 EUR (confidential discounts are not captured).
- Prices and target populations were summarised using medians and interquartile ranges (IQR).
- The association between launch price and target population size was assessed using Spearman rank correlation coefficients.
- Reimbursement outcomes were classified based on HTA decisions.
- Exploratory budget exposure estimates used public launch prices and target population estimates. Reimbursed and non-reimbursed therapies were compared using a two-sample t-test.

Results

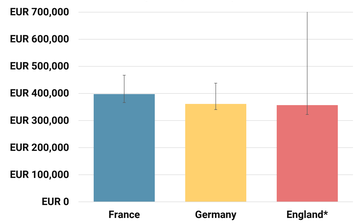
- A total of 73 HTA reports covering 17 products were included, with both HTA-derived target population estimates and publicly available launch price data.
- Launch prices demonstrated an inverse relationship with target population size across all markets, supporting the application of rarity-weighted pricing principles.
- This relationship was strongest in England (ρ = -0.51), moderate in Germany (ρ = -0.39), and weaker in France (ρ = -0.22).
- Median launch prices were EUR 397,680 in France (IQR: 366,450–467,334), EUR 361,860 in Germany (IQR: 340,554–437,958), and EUR 356,428 in England (IQR: 322,435–1,978,510), despite differences in assessed population size.
- Reimbursement outcomes were not consistently aligned with price level: several high-cost therapies (>EUR 2M) were reimbursed, while lower-cost oncology therapies were not reimbursed in certain markets.
- Non-reimbursed therapies showed higher median exploratory budget impact estimates than reimbursed therapies (EUR 187M vs EUR 132M); however, this difference was not statistically significant (p=0.87), suggesting that reimbursement decisions reflect considerations beyond financial impact alone.

Inverse relationship between population size and launch price across markets



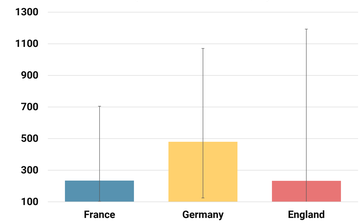
Log scale: 1=10 patients, 2=100, 3=1,000, 4=10,000

Median launch prices across markets (EUR, 2025)

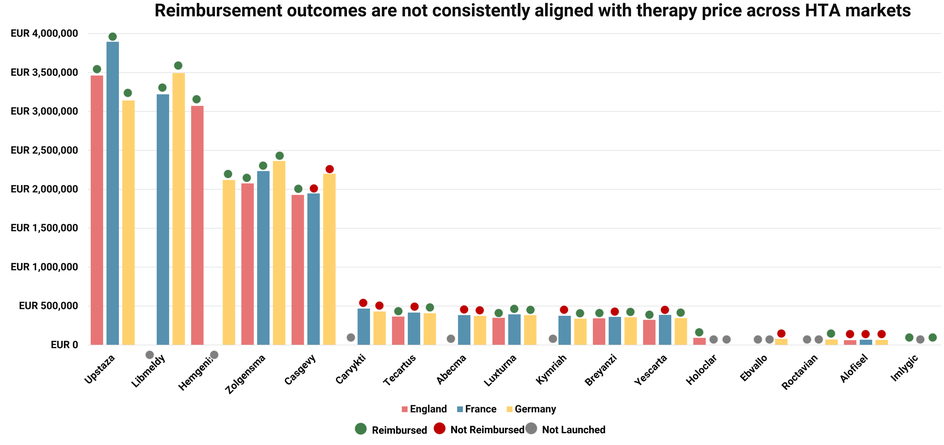


**England upper quartile exceeds displayed axis range due to extreme value*

HTA-derived target population sizes across markets (median and IQR)



Reimbursement outcomes are not consistently aligned with therapy price across HTA markets



Conclusions

- Launch prices for advanced therapies are higher in smaller target populations, consistent with rarity-weighted value-based pricing observed across HTA-driven markets.
- Reimbursement outcomes are not solely driven by price or exploratory budget impact estimates, but reflect broader considerations including clinical uncertainty, unmet need, and HTA framework-specific decision rules.
- Cross-country variation highlights the role of national HTA systems in shaping both pricing and access, supported by the use of HTA-derived population estimates to assess value and affordability.
- In contrast, the absence of a centralised HTA framework in the US limits comparability and reduces the use of structured population-based value assessments.
- As most-favoured-nation (MFN) pricing gain traction, linking US prices to HTA-based benchmarks may have unintended consequences, including downward pressure on global prices and altered launch sequencing, particularly for rare and ultra-rare therapies.

References

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2. Angelis, A., Naci, H., & Hackshaw, A. (2020). Recalibrating health technology assessment methods for cell and gene therapies. Pharmacoeconomics, 38(12), 1297-1308.
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Abbreviations

HTA, Health Technology Assessment; CGTs, Cell and Gene Therapies; G-BA, Federal Joint Committee (Gemeinsamer Bundesausschuss); HAS, French National Authority for Health (Haute Autorité de Santé); NICE, National Institute for Health and Care Excellence; IQR, Interquartile Range

Acknowledgements and Contact Information

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