DISPARITIES IN MARKET ACCESS OF ATMPS: A COMPARATIVE ANALYSIS OF HTA OUTCOMES ACROSS EUROPE

HTA113

Steven Horsburgh[1], Audrey E Fulthorp[1], Georgia Hollier-Hann[1], Stephen Ralston[1]

[1]Coronado Research, Newcastle upon Tyne, England

INTRODUCTION

- Advanced Therapy Medicinal Products (ATMPs - gene therapies and cell/tissue therapies) offer transformative potential for treating rare and life-threatening diseases.
- Health technology assessment (HTA)/reimbursement processes were established based on chronic therapies, therefore, high cost ATMPs which are administered once with potential life-long benefits can be concerning for payers due to the strain that funding these therapies can place on healthcare budgets.[1]
- The availability of these therapies often differs between US and Europe due to differences in how these markets work;[2] EMA or MHRA marketing authorisation may not translate to patient access due to individual member state HTA requirements.
- Patient access to these therapies may also vary within Europe due to differences in national HTA body mechanisms and manufacturer strategies; access discrepancies have meaningful implications for patients with rare diseases who may miss out on potentially curative treatments.

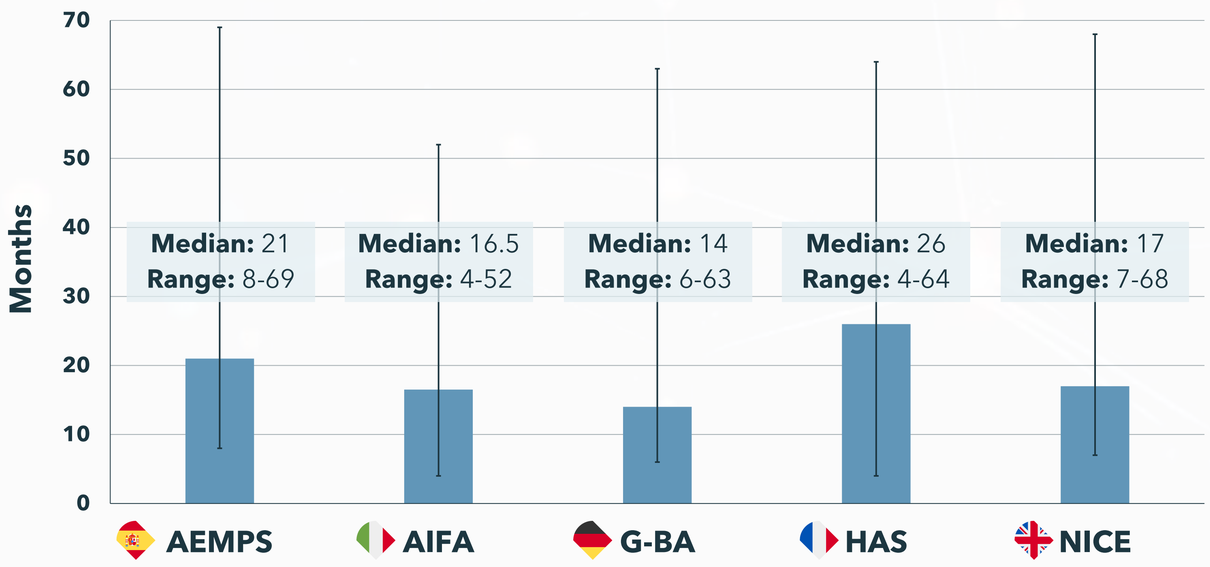
OBJECTIVE AND METHODS

- The objective of this study was to explore disparities in approval outcomes in Europe.
- ATMPs were identified through targeted literature searches, and those with EMA marketing authorisation were identified via the EMA website;[3] no time restrictions were put in place therefore all currently approved ATMPs were included.
- Searches were conducted to identify HTAs and associated documentation for HTA bodies NICE (England and Wales), G-BA (Germany), HAS (France), AIFA (Italy) and AEMPS (Spain).
- Key outcomes included whether an assessment was conducted, frequency of positive outcomes, time from EMA approval to HTA publication, and the use of conditional access mechanisms.

RESULTS

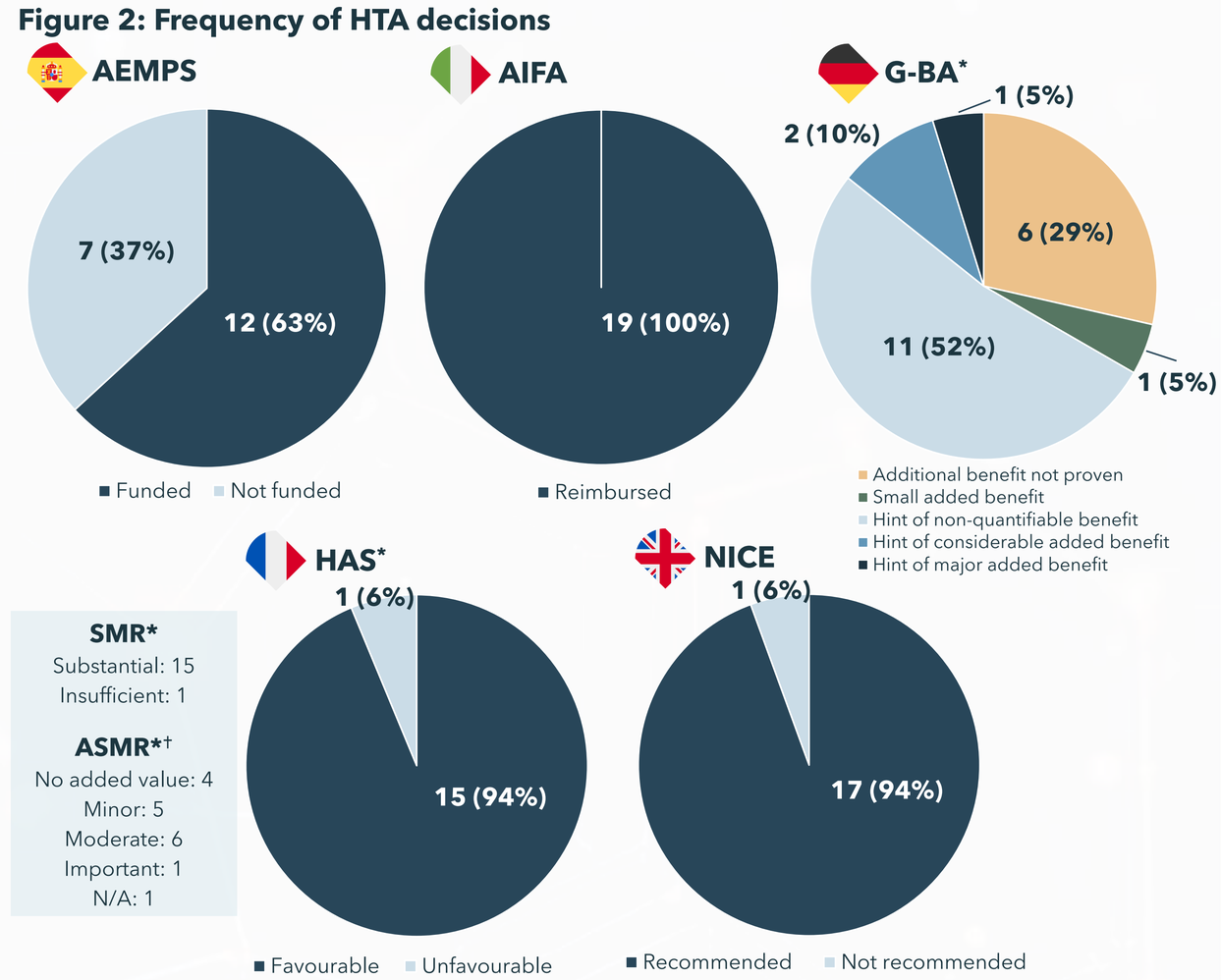
- Of the 18 ATMPs currently with EMA marketing authorisation, eight are gene therapies, six are CAR-T therapies, and four are other cell therapies.
- Seven (39%) are oncology treatments and 11 are for non-oncology indications (61%). At the time research was conducted, 14 (78%) of the ATMPs had active EMA orphan designation, while two had orphan status withdrawn.
- AIFA assessed all 18 (100%) ATMPS, AEMPS and NICE assessed 14 (78%), G-BA assessed 12 (67%), and HAS assessed nine (50%).
- The median duration from EMA marketing authorisation to publication of the HTA decision varies between the countries; however, there is a large range in time to HTA decision in all countries (Figure 1).

Figure 1: Median (± range) time from marketing authorisation to HTA decision



- G-BA conducted 20* HTAs, AIFA and AEMPS both conducted 19, NICE conducted 18 and HAS 15* (Figure 2).

Figure 2: Frequency of HTA decisions



- NICE recommended three drugs (17%) through the CDF, HAS granted early access authorisation to six drugs (38%), while AIFA granted four drugs (22%) innovation status and two (11%) were reimbursed through managed entry agreements ("payment by results").

CONCLUSION

- These data show that reimbursement of ATMPs differs amongst key European markets, both in terms of which ATMPs have been assessed and the frequency of positive outcomes. Positive outcomes were more frequent than negative decisions in all countries assessed; however, the number of ATMPs/HTAs varied.
- Conditional access mechanisms such as managed entry agreements were not utilised in most cases; therefore, there may be scope to adapt assessment methods further to allow greater flexibility in conditional access for rare diseases with unmet need while additional data are collected.
- In terms of time from marketing authorisation to HTA decision, there was a large range in each country, suggesting that the time to HTA decision could be due to complexities of the drug, indication, and/or evidence package rather than any one country/HTA body being inherently quicker.
- With the introduction of JCA in January 2025, the aim is to facilitate more consistent decision-making across Europe and to help "timely decisions when bringing medicines to the market", thereby reducing disparities.[4]
- These findings demonstrate the importance of timely and tailored market access strategies to optimise patient access across Europe without unnecessary delays.

Footnotes: *Libmeldy counted twice due to two decisions from a single HTA (Late infantile or early juvenile forms without clinical manifestations and early juvenile forms with early clinical manifestations); †Zolgensma counted twice due to different ratings for subgroups.

Abbreviations: AEMPS, Agencia Española de Medicamentos y Productos Sanitarios (Spanish Agency for Medicines and Health Products); AIFA, Agenzia Italiana del Farmaco (Italian Medicines Agency); ASMR, Amélioration du Service Médical Rendu (improvement in actual benefit); ATMP, Advanced Therapy Medicinal Products; CDF, Cancer Drugs Fund; EMA, European Medicines Agency; HAS, Haute Autorité de Santé; HTA, health technology assessment; G-BA, Gemeinsamer Bundesausschuss (Joint Federal Committee); JCA, Joint Clinical Assessment; NICE, National Institute for Health and Care Excellence; SMR, Service Médical Rendu (actual benefit).

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