PAYING FOR CURES: AN ANALYSIS OF INNOVATIVE ACCESS AGREEMENTS FOR LICENSED GENE THERAPIES

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BACKGROUND

Gene therapies are often one-time curative treatments that address the underlying cause of disease, helping patients avoid a lifetime of therapy, while saving health systems the associated resource burden. Gene therapies are therefore able to command high-price premiums. As a result, health systems now face the challenge of how to sustainably provide equitable patient access to this growing class of advanced, innovative therapeutics.

OBJECTIVES

To assess the reimbursement status and access agreements in place for reimbursed gene therapies in the European Union (EU) and the United States (US).

METHODOLOGY

The research reviewed all gene therapies approved by the European Commission (EC) or US Food & Drug Administration (FDA), and with a positive or conditional national reimbursement status in England, France, Germany, Italy or Spain. Using the respective country healthcare technology assessment (HTA) websites in September 2019. This was supplemented with a targeted literature review of the associated manufacturer press releases and an open internet search using keywords: Imlygic, Strimvelis, Kymriah, Yescarta, Luxturna, Zynteglo, Zalmoxis, Zolgensma.

RESULTS

There are seven licensed gene therapies in the EU and five in the US[1,2]. Five are reimbursed in England, three in Germany and Italy and two in France and Spain[3,4,5,6,7]. The list prices range from €1,837 per vial for Imlygic in England, which requires continuous treatment for a minimum of six months, up to €1,890,000 per patient for Zolgensma in the US requiring a single dose[8,9] (figure 1).

FIGURE 1: LIST PRICE (€)* OF REIMBURSED GENE THERAPIES [5, 8, 9, 10, 11, 12]

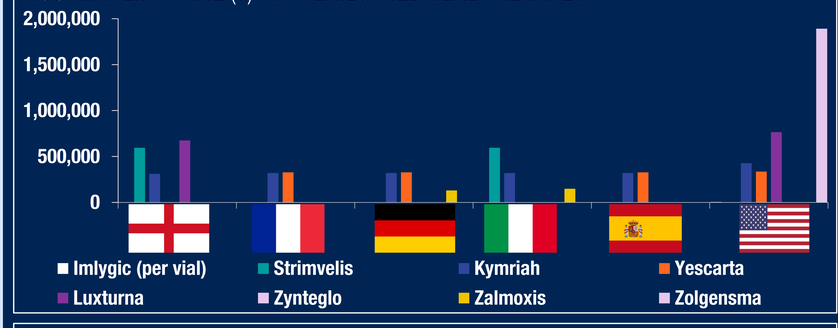

FIGURE 2: ACCESS AGREEMENTS [2, 3, 13, 14, 15, 16, 17, 18, 19, 20]

Gene therapy	England	France	Germany	Italy	Spain	USA
Imlygic	Simple discount & PAS	Not submitted	Not reimbursed	Not reimbursed	Not reimbursed	5-year patient follow up
Strimvelis	None	Not submitted	Not submitted	Outcome-based refund & drug-leasing over 5-years	Not submitted	Not licensed
Kymriah	Confidential discount; 5-year additional data collection; funded by CDF	Early access programme	Outcome-based refund	Outcome-based refund & drug-leasing over 5-years	Outcome-based refund & drug-leasing over 5-years	15 year patient follow-up; funded by Medicare
Yescarta	Confidential discount; 3-year additional data collection; funded by CDF	Early access programme	Outcome-based refund	Not submitted	Outcome-based refund & drug-leasing over 5-years	15 year patient follow-up; funded by Medicare
Luxturna	Confidential discount	Pending	Pending	Pending	Not submitted	Outcome-based rebate; drug-leasing under discussion
Zynteglo	Not submitted	Not submitted	Not submitted	Not submitted	Not submitted	Not licensed
Zalmoxis	Not submitted	Not reimbursed	None	None	Not submitted	Not licensed
Zolgensma	Not licensed	Not licensed	Not licensed	Not licensed	Not licensed	Outcome-based refund & drug-leasing over 5-years

Access agreements include discounts, outcome-based rebates/refunds if patient outcomes fail to meet pre-specified thresholds and "drug leasing", which involves payment by instalments, typically over five years (figure 2).

Discounts were agreed for the majority of reimbursed gene therapies in England[3]. Outcome-based rebates/refunds were agreed for the majority of reimbursed gene therapies in Germany and Italy and for both of the reimbursed gene therapies in Spain[5,14,15,16,17]. In the US, outcome-based rebates/refunds were agreed for Luxturna and Zolgensma, which have the highest list prices of the licensed gene therapies at €765,000 and €1,890,000 respectively[9,19,20]. Drug leasing was agreed for the high-cost CAR-T therapies in Spain, for Kymriah and Strimvelis in Italy and for Zolgensma in the US, with Luxturna still under discussion[5,15,16,17,19,20].

Long-term real-world evidence (RWE) collection was also a common requirement, either to resolve clinical uncertainties in safety and effectiveness – such as Imlygic in the US and Kymriah and Yescarta in England and the US – or to generate additional data to inform reimbursement decisions of gene therapies with outcome-based agreements[2,3].

CONCLUSIONS

Most gene therapies that do not address ultra-orphan diseases are subject to innovative access agreements and long-term RWE collection programmes as a condition for reimbursement. These schemes demonstrate health systems' efforts to minimise clinical uncertainties, manage high treatment costs, and inform HTA decisions.

DISCUSSION

Gene therapy manufacturers are encouraged to engage early with country pricing and reimbursement authorities, to explore innovative market access approaches that enable health systems to balance patient access against any potential risks.

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*Exchange rate used (£GBP1 : €1.1; $US1 : €0.9);

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