# Evaluating Key Drivers for Patient Access and Pricing Trends in ATMPs across Germany, France, and the UK

**EMILY NG, MSc – SENIOR ANALYST, MITUN PATEL, MSc - DIRECTOR**
**RED NUCLEUS, MARKET ACCESS AND COMMERCIALISATION SERVICES, LONDON, UK**

## OBJECTIVES

With the rapid development of Advanced Therapy Medicinal Products (ATMPs), managing the budget impact of introducing these high-cost therapies poses a key challenge for healthcare systems. Payers often restrict access to high-cost therapies due to uncertainties in clinical evidence, and to balance budgets and maintain healthcare sustainability. By examining ATMPs launched in Germany, France, and the UK over the past eight years, this analysis focuses on Luxturna as a case study to understand how clinical trial packages influence health technology assessment (HTA) reimbursement/pricing outcomes.

## METHODS

ATMPs granted market authorisation between 2015 and 2023 were identified from European Medicines Agency (EMA) and Medicines and Healthcare Products Regulatory Agency (MHRA) databases. Data on ATMP HTAs, including presented clinical trial evidence, and pricing and reimbursement decisions, were gathered from relevant HTA bodies across Germany, France, and the UK.

Product data were then compiled in a spreadsheet, categorised by disease characteristics (e.g. prevalence, age group, and on-label competitors), product attributes (e.g. EMA indication, line of therapy, administration route), trial details (e.g. trial type, comparator, and endpoints), as well as pricing and reimbursement outcomes (e.g. HTA rating, reimbursement levels and annual therapy costs).

**Launch Overview of ATMPs (2015-2023)**

| Product Name | Launch Date | Orphan Drug Designation | Therapeutic Area |
|---|---|---|---|
| **Hemgenix** | 2023 | No | Haematology |
| **Upstaza** | 2022 | Yes | Neurology |
| **Roctavian** | 2022 | Yes | Haematology |
| **Ebvallo** | 2022 | Yes | Oncology |
| **Zolgensma** | 2022 | No | Neurology |
| **Carvykti** | 2022 | Yes | Oncology |
| **Breyanzi** | 2022 | No (status withdrawn) | Oncology |
| **Abecma** | 2021 | Yes | Oncology |
| **Skysona** | 2021 (product withdrawn) | Yes | Neurology |
| **Libmeldy** | 2020 | Yes | Neurology |
| **Tecartus** | 2020 | Yes | Oncology |
| **Luxturna** | 2018 | Yes | Ophthalmology |
| **Yescarta** | 2018 | Yes | Oncology |
| **Alofisel** | 2018 | No | Immunology |
| **Spherox** | 2017 | No | Orthopaedic |
| **Imlygic** | 2015 | No | Oncology |
| **Holoclar** | 2015 | No | Ophthalmology |

## RESULTS

Of the 17 ATMPs with regulatory approval across selected markets, 10 had Orphan Drug Designation and 7 were in the oncology disease area. Most of these therapies address diseases with significant impacts on mortality and are associated with chronic, long-term progression.

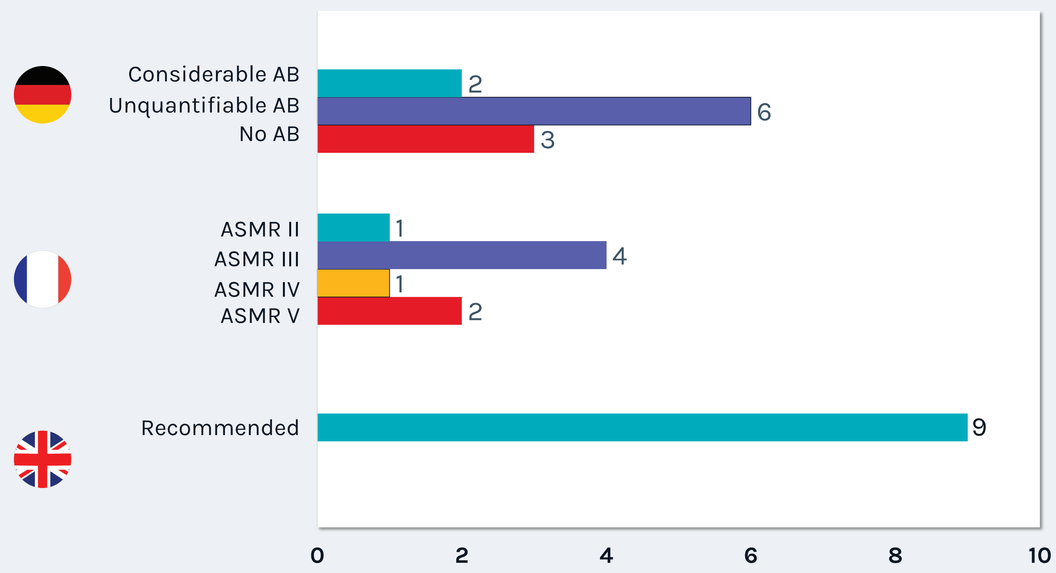
In Germany, only Luxturna and Tecartus achieved a 'considerable added benefit rating'. Luxturna, used to treat vision loss from inherited retinal dystrophy, is supported by a randomized phase 3 trial and an observational study assessing long-term safety and efficacy. Key findings include statistically significant and clinically meaningful improvements in functional vision (MLMT), light sensitivity (FST), and quality of life, with no mortality reported. The long-term follow-up confirms these benefits, categorizing its overall impact as considerable.

In France, Luxturna is the only product to receive an 'SMR Important' and 'ASMR II' rating. Following a similar HTA evaluation as in Germany, the Haute Autorité de Santé (HAS) noted that the efficacy of existing treatments, such as Vitamin A palmitate and lutein-DHA, is subject to debate, and that current medical devices do not target the same patient population. Luxturna was rated as providing high clinical benefit, with significant improvement in functional vision in both eyes one-year post-treatment.

In the UK, NICE has recommended Luxturna along with eight other ATMPs. Recognising the rarity and severity of the disease, NICE found that clinical trial data showed short-term vision improvements, with the plausible assumption that these benefits could extend over decades, despite the lack of long-term evidence. While economic modeling uncertainties remain, particularly around utility values and treatment duration, NICE concluded that Luxturna likely offers significant clinical benefits for patients.

Pricing for Luxturna is relatively consistent across Germany (€295K annually), France (€290K), and the UK (£269K). In contrast, Upstaza received an 'unquantifiable added benefit rating' in Germany, 'SMR Important / ASMR III' in France, with an annual cost of €3M and €3.5M respectively, and is recommended in the UK at €2.6M annually. This pricing discrepancy highlights the UK's emphasis on cost-effectiveness and variability in acceptance for single-arm, on-going trial studies.

### Overview of HTA Outcomes for ATMPs




*Products that have not been launched in the country, are part of an early access program, or are still undergoing HTA are excluded from the analysis.*
*Abbreviations: AB: Additional Benefit*

## CONCLUSION

The analysis highlights the significant variability in HTA outcomes and pricing for ATMPs across Germany, France, and the UK. These findings emphasise the importance of trial design, including factors like randomisation, open-label, patient-relevant endpoints, in influencing payers' willingness to pay. Understanding these key drivers is crucial for assessing the robustness of trial evidence required to achieve a certain level of added clinical benefit. This knowledge informs pricing strategies and highlights the additional evidence needed to secure optimal HTA reimbursement and expedite patient access.

## REFERENCES

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Author contact details: **Emily Ng**
email: eng@rednucleus.com
www.rednucleus.com/macs