Innovative Reimbursement Agreements Addressing the Challenges of Advanced Therapy Medicinal Products: Systematic Review for the Five Major European Healthcare Systems (EU5)

HPR244

Roman Spelsberg [1,2], Leonard Trappe [3], Mark J. G. Govers [1], Silvia M. A. A. Evers [1]

[1] Department of Creating Value Based Healthcare CAPHRI, Maastricht University, Maastricht, Netherlands
[2] Healthcare Research & Market Access, fbeta GmbH, Berlin, Germany
[3] Life Sciences & Chemistry, High-Tech Gruenderfonds, Bonn, Germany

INTRODUCTION

Advanced therapy medicinal products (ATMP) aim to deliver transformative effects in the treatment of severe and orphan conditions.

Their high short-term budget impact and associated uncertainties regarding their long-term efficacy pose challenges for reimbursement.

METHOD

The primary objective of this systematic review was to gain insight into the types of reimbursement agreements used for the funding of ATMPs in the statutory health insurance systems across the five major European countries (EU5).

Medline, Embase, Scopus, Web of Science and national HTA websites were systematically searched.

Conclusion

In contrast to other disruptive health innovations, such as digital therapeutics (DTx), payers across the EU5 can assess the value of ATMPs and inform reimbursement decisions with minor adjustments of established pathways.

However, the individual reimbursement agreements for ATMPs went beyond traditional drug pricing mechanisms.

The urgency for ATMP adoption led to temporal decoupling of reimbursement and completed evidence generation.

Managed entry agreements linking the reimbursement of ATMPs to their outcomes in the clinical practice address their challenges and have the potential to enable sustainable market access to these promising innovations.

Results

43 records, and 141 HTA reports published until 24/08/2023 were included in the synthesis.

France and the UK developed CED mechanisms to collect RWE for a reassessment of the ATMPs.

Spain and Italy introduced outcomes-based staged payments. These agreements split the payment from the payer to the MAH in separate instalments. Each instalment is only initiated, if the ATMP reaches and maintains positive outcomes.

In Germany and Italy outcomes-based risk-sharing agreements have been negotiated. A refund from the MAH to the payer will be initiated if the expected treatment outcomes are not reached.

Classification	Brand name	Germany	France	Italy	Spain	UK excl. SCO
GTMP	Imlygic®	+	-	-	-	±, FA
	Strimvelis®	IPTR	-	+, PBA	-	+
	Kymriah®	+, PBA	+, CED	+, PBA	±, PBA	+, CED
	Yescarta®	+, PBA	+, CED	+, PBA	±, PBA	+, CED
	Luxturna®	+, FA	+, CED	+	+, PBA	±, FA
	Zolgensma®	+, CED, PBA	+, CED	±	±, PBA	+, FA
	Libmeldy®	+	±	±	-	+, FA
	Abecma®	+	±, CED	Ongoing	-	-
	Tecartus®	+, CED	+	±	Ongoing	+, CED
	Breyanzi®	+	±, CED	Ongoing	Ongoing	-
	Carvykti®	+	±, CED	-	-	-
	Upstaza®	+	±, CED	±	-	+, FA
	Roctavian®	+	IPTR	Ongoing	-	Ongoing
	Hemgenix®	+	+, CED	Ongoing	-	Ongoing
sCTMP	Alofisel®	+, PBA	±	-	±, PBA	-
	Ebvallo®	+, FA	±, CED	-	-	-
TEP	Holoclar®	+	±	+, PBA	-	±, FA
	Spherox®	-	-	-	-	±
Number of reimbursed ATMPs		17	15	9	5	11

ATMP, advanced therapy medicinal product
CED, coverage with evidence development
FA, financial-based managed entry agreements including patient access schemes with simple discounts and flat price per patient schemes
GTMP, gene therapy medicinal product
HTA, health technology assessment
IPTR, individual patient treatment request
MAH, Marketing authorization holder
Ongoing, HTA on-going and final reimbursement decision pending
PBA, performance-based managed entry agreements including outcomes-based risk sharing and outcomes-based staged payments
RWE, Real World Evidence
sCTMP, somatic cell therapy medicinal product
TEP, tissue-engineered medicinal product
UK, United Kingdom
+ National reimbursement or positive reimbursement recommendation without restrictions
± National reimbursement with restrictions on certain indications or patient population
- Negative reimbursement decision, negative reimbursement recommendation, not (yet) commercialized

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CONTACT INFORMATION

Roman Spelsberg
roman.spelsberg@maastrichtuniversity.nl
roman.spelsberg@fbeta.de
+49 171 303 4736