Unlocking Equitable Access to Revolutionary Cell and Gene Therapies Across EU Member States

HTA44

View all Parexel's posters at ISPOR Europe 2024

Dalton, Ronan; Perez-Kempner, Lucia; Budhia, Sangeeta

Background

Cell and gene therapies (C&GTs) represent the cutting-edge of modern medical innovative and have been developed to address the needs of patients across certain severe conditions. However, C&GTs are among the most expensive medical interventions currently available. Across the European Union (EU) marketing authorization (MA) may be granted centrally. However, health technology assessments (HTA) and decisions on reimbursement are usually made at the national level by the individual EU Member States. Despite efforts to align the assessment process, national assessment criteria and techniques still vary widely, as do payer decisions, leading to unequal access across the EU and thus health inequities between citizens. The objective of this research was to analyze the access landscape for C&GTs across a range of EU Member States and identify if any inequities in access exist, and, if so, what reduction strategies could be considered to improve access to care moving forward.

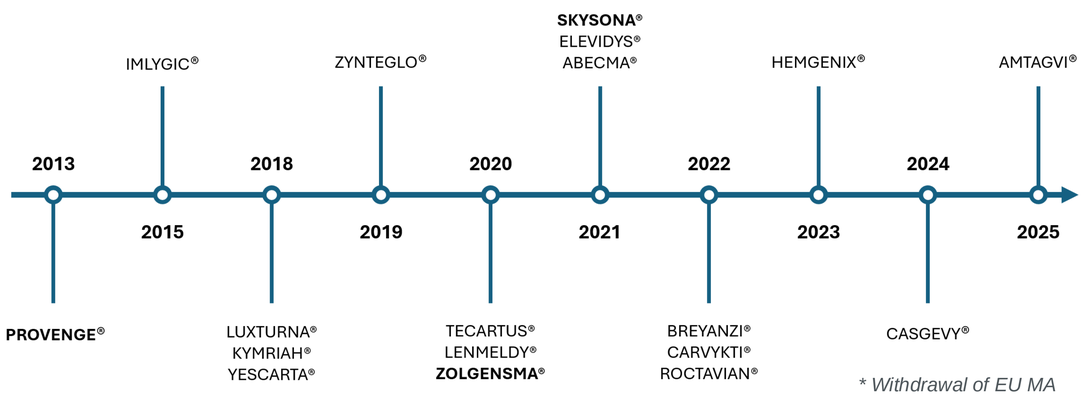
Methods

The European Medicines Agency (EMA) database was searched to identify C&GTs since first approval. The National HTA reports for the identified therapies were reviewed for a selection of large and small/mid-sized Member States, including France (HAS), Germany (G-BA), Greece (EOT), Ireland (HPRA), Italy (AIFA), Poland (AOTMiT), and Sweden (TLV). Therapeutic positioning reports (IPTs) were reviewed in Spain. HTA reports from the English HTA agency (NICE) were included despite England no longer being an EU member state. Comparisons of recommendations were made based on evidence considered (i.e., clinical evidence vs economic assessment).

Results

Since 2013 17 C&GTs have been granted EU MA. As of 2024, 14 remain on the market (**Figure 1**).

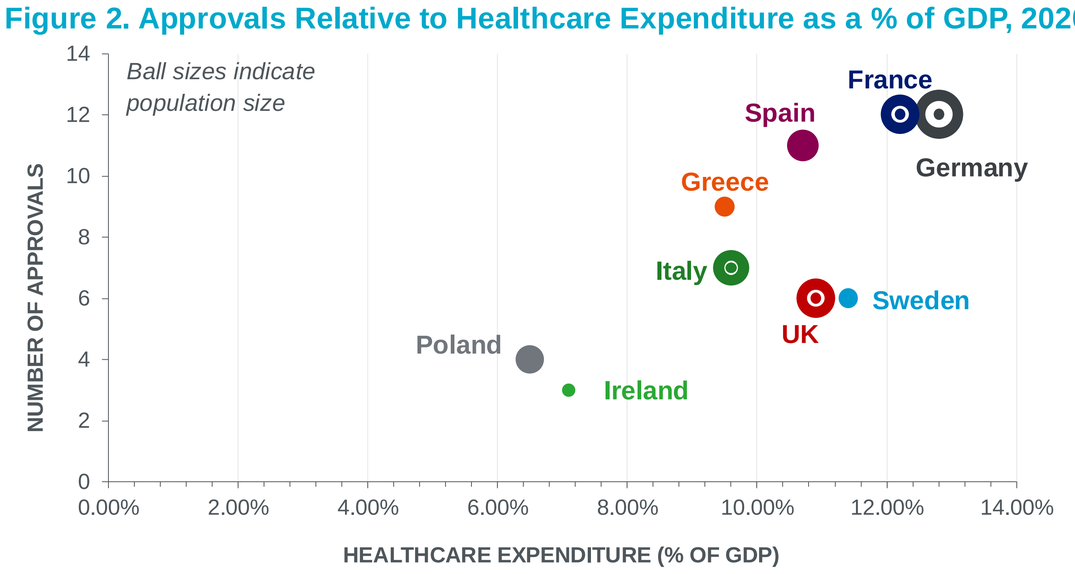
Figure 1. Timeline of EU MA for C&GTs



** Withdrawal of EU MA*

With a focus on C&GTs still under commercialization in the EU, a search of the national HTA agencies identified 78 individual HTA national decisions (**Table 1**). The number of reimbursed products was generally higher in Member States where healthcare expenditure relative to gross domestic product (GPD) (**Figure 2**) was higher.

Figure 2. Approvals Relative to Healthcare Expenditure as a % of GDP, 2020



Countries listed by approvals (Healthcare spending as a % of GDP, size of balls relative to population size): Germany: 12 (12.8% - ~83 million people); France: 12 (12.2% - ~68 million people); Spain 11 (10.7% - ~48 million people); Greece: 9 (9.5% - ~10 million people); Italy: 7 (9.6% - ~59 million people); Sweden: 6 (11.4% - ~10 million people); UK: 6 (10.9% - ~67 million people); Poland: 4 (6.5% - ~37 million people); Ireland: 3 (7.1% - ~5 million people)

Table 1. HTA Decisions Regarding C&GTs in Selected EU Member States

C&GT	Payer Decision								
	NICE	HAS	G-BA	EOPYY	HPRA	AIFA	AOTMiT	AEMPS	TLV
IMLYGIC®	R	NE	R	NE	NR	R	NE	R	NE
LUXTURNA®	R	R	R	R	R	R	NR	R	R
KYMRIAH®	T	R	R	R	NA	R	R	R	R
YESCARTA®	R	R	R	R	NA	NE	R	R	R
TECARTUS®	R	R	R	R	NA	R	R	R	R
LIBMELDY®	R	R	R	NE	R*	NE	NE	NE	R
ZOLGENSMA®	R	R	R	R	R	NE	R	R	R
ABECMA®	T	R	R	R	NE	R	NR	R	NR
ELEVIDYS®	NA	NE	NE	NE	NE	NE	NE	NE	NE
BREYANZI®	NA	R	R	R	NE	R	NR	NA	NA
CARVYKTI®	T	R	R	R	NA	NA	NR	R	NR
ROCTAVIAN®	NA	R	R	NE	NE	NA	NR	R	NA
HEMGENIX®	NA	R	R	R	NE	R	NR	R	NA
CASGEVY®	NA	R	NA	NE	NE	NE	NE	R	NE

AEMPS: Agencia Española de Medicamentos y Productos Sanitarios; AIFA: Agenzia Italiana del Farmaco; AOTMiT: Agencja Oceny Technologii Medycznych i Taryfikacji; C&GT: Cell & gene therapies; EOPYY: National Organization for Health Care Services Provision; EU: European Union; G-BA: Gemeinsame Bundesausschuss; HAS: Haute Autorité de Santé; HPRA: Health Products Regulatory Authority; HTA: Health technology assessment; TLV: Andvårds-och läkemedelsförmånsverket; NA: Not available, because a decision on reimbursement has not been reached yet; NE: Not evaluated; R: Reimbursed

R REIMBURSED | NR NOT REIMBURSED | NA REIMBURSEMENT DECISION PENDING | NE NOT EVALUATED

Positive reimbursement was more frequently observed in those countries where only clinical-effectiveness was evaluated. In line with this, favorable reimbursement rates >75% were observed in France (12/14), Germany (12/14), and Spain (11/14). In the contrary, lower reimbursement rates were observed in countries where economic evaluations were also performed as part of the evaluation process, ranging from 64% (9/14) in Greece to 21% (3/14) in Ireland. In the case of Ireland, reimbursement has been supported by the country joining the BeNeLuxa initiative in 2021, which has supported the reimbursement of LIBMEDLY and ZOLGENSMA through a facilitated joint assessment process.

Conclusions

- Access to C&GTs is highly variable across the EU, especially in small/mid-sized Member States who operate under models of reimbursement that include the use of economic evaluations, when compared to larger Member States who operate under a clinical effectiveness model of reimbursement. In those small/mid-sized Member States reviewed, who focus on an economic evaluations in their reimbursement process, healthcare spending relative to GDP tend to be lower than the EU average. This, coupled with the high per-capita costs, due to small target populations, and limited long-term efficacy data, is pushing cost-effectiveness above the accepted thresholds, thus discouraging risk-taking, and thereby limiting access. Conversely, Member States who operate under a clinical effectiveness model of reimbursement tend to have increased healthcare spending relative to GDP, prioritize therapeutic benefits even with limited long-term efficacy data, and, therefore, facilitated access to innovative therapies.
- The introduction of the EU Joint Clinical Assessment regulation will provide streamlined, standardized and clinical assessment of innovative medications. This, in combination with managed entry agreements and innovative pricing strategies, and membership of joint procurement cooperations (as demonstrated by the successful BeNeLuxA negotiations for LIBMELDY and ZOLGENSMA) may support price discounts and the achievement of cost-effectiveness in mid-sized Member States, increasing access to innovative therapies and helping to reducing health inequities across Europe.

REFERENCES

[1] European Medicines Agency: Medicines for human use.

[2] Eurostat. Healthcare expenditure statistics: Current healthcare expenditure relative to GDP, 2020.

[3] BENELUXA. BENELUXA Initiative announces successful conclusion of Libmeldy price negotiations, directly benefiting patients in three countries. 2024

[4] BENELUXA. Outcome of joint negotiations for Zolgensma.2021