

P&R Policies and Macro-Trends Pertinent to CGTs: Where Are the Main Barriers and Who Leads the Efforts to a Greater Access?

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Objectives

Cell and gene therapies (CGTs) are a rapidly growing therapeutic area holding great promise for treating diseases with high unmet medical needs. Nearly 60% of the 2,400 ongoing CGT trials at the end of 2021 targeted prevalent diseases, a clear indication of the sector's evolution. The intention of our paper is to highlight where recent policy developments suggest the most promising or challenging outlook.

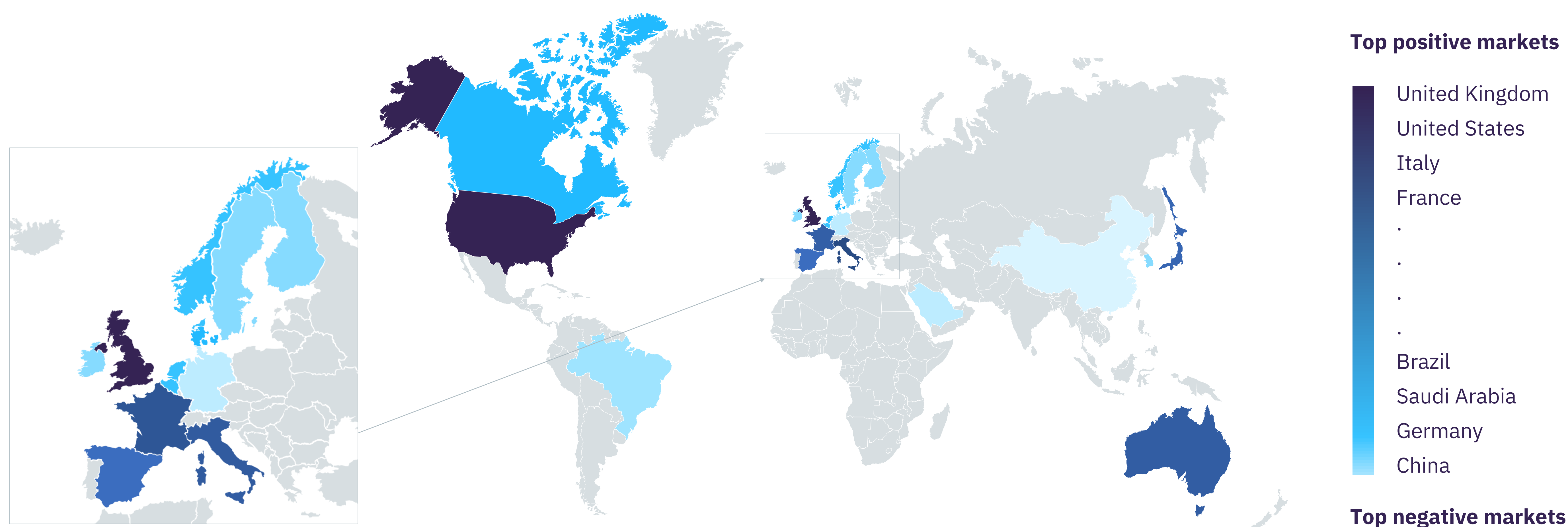
Methods

An extensive screening of 2021 news and reports was performed for 20 markets spanning five regions, selected as a representative sample of the global pharma sector. Official sources and relevant media in local languages were consulted. Selected events covered healthcare policy enacted/proposed reforms, discussions around pricing and reimbursement (P&R) adaptation, trade associations and patient organizations advocacy, regulatory approvals, P&R decisions, and HTA opinions on nine key CGTs. Implications and outlook were analyzed and classified into positive, neutral/mixed, or negative depending on the anticipated impact on multinational manufacturers.

Results

A total of 159 significant developments were selected and analyzed, with **57%** of these relating to the **nine key therapies** in scope:

Abecma Breyanzi Alofisel Kymriah Libmeldy Luxturna Tecartus Zolgensma Zynteglo



The US and the EU countries generally present positive and neutral events, with Germany being an outlier where considerable pressure has recently been put on price and access conditions due to growing focus on stricter cost containment measures.

82 developments had positive implications with the UK, US, and Italy accounting for 17%, 16%, and 10%, respectively. 40 negative developments were identified overall, and 37 entailed neutral/mixed implications. The highest number of negative events were registered in Germany and China, accounting for 18% and 13%, respectively.

Although four positive events were observed in Canada that pertain to the nine key analogs, three negatives were also detected, indicating a challenging environment where P&R approach towards CGTs is still not well structured. While the Canadian Agency for Drugs and Technologies in Health (CADTH) in multiple cases required a discount of at least 90% of proposed price to issue a positive recommendation on reimbursement, the pan-Canadian Pharmaceutical Alliance (pCPA) has proven effective in supporting market access across the country. Likewise, a similar ratio of positive and negative incidents can be observed in South Korea and Japan. A high number of negative events were registered in China, with no CGTs approved for reimbursement so far, although it appears to be moving towards a more dynamic reimbursement process, with the time between the National Reimbursement Drug List updates having shortened from seven years to one year. Even though core pricing policies towards CGTs are still unfavorable in emerging markets, efforts are being made to ease access.

Conclusions

Despite budget constraints and higher uncertainties, several markets are making efforts to improve market access conditions for CGTs, especially in the EU as apparent by the high number of positive events in this analysis. The UK and Italy are relatively open to innovation and remain attractive markets with a variety of supporting tools. The US prioritizes CGTs' development and expedited access, with increasing calls for value-based purchasing arrangements. It also remains the most liberal pricing environment for innovative drugs globally. On the other hand, Germany is adding considerable pressure on high-cost medicines threatening its early-launch market attractiveness, while China presents important challenges for imported overseas products by favoring domestic production. Our analysis looked primarily at ongoing trends, and a limitation here is that each country has a different baseline.