Characteristics of Cell and Gene Therapies Authorized by the Food and Drug Administration and the European Medicines Agency

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Background

Cell and gene therapies represent a promising shift in the treatment and prevention paradigm for cancer, genetic diseases, and other conditions lacking effective treatments.

Objective

This study examined the characteristics of the authorizations of cell and gene therapies by the European Medicines Agency (EMA) and the Food and Drug Administration (FDA).

Methods

Gene and cell therapy data were gathered from the FDA and EMA websites up until December 31, 2023. The data was analyzed descriptively.

The focus of the analysis was identifying patterns in regulatory decisions, including orphan designations, and expedite review designations and processes, and assessing the time gap between approvals by the FDA and EMA

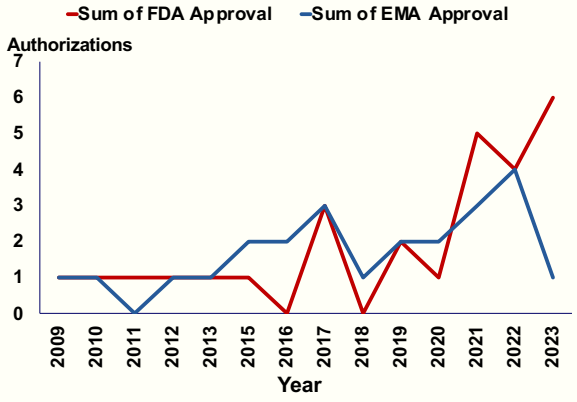
Results

The FDA and EMA authorized 21 gene and 14 cell therapies. The FDA authorized 26 and the EMA 24. The first 2 therapies were authorized in 2009, 15 therapies (1.5±0.7 year) in 2010-2019, and 19 (4.8±1.9) in 2020-2023. 15 products were approved by both agencies, with 12 approved first by the FDA and a median EMA approval gap of 145 days (interquartile range 280 days). The EMA withdrew authorization for 3 gene and 4 cell therapies.

The therapeutic classes with the largest number of authorizations were antineoplastic and immunomodulating agents (4, 40.0%), blood and blood-forming organs (6, 17.1%), alimentary tract and metabolism (4, 11.4%), and musculoskeletal system (4, 11.4%).

Results

Figure 1. Cell and Gene Therapy Authorized by the FDA and the EMA (2009-2023)

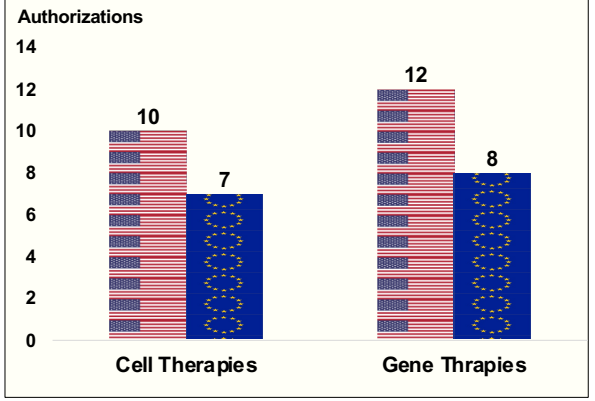


Orphan designation was granted to 22 (84.6%) therapies authorized by the FDA and 15 (62.5%) by the EMA. There were 28 (80%) that had orphan designation in both agencies.

The FDA used priority review for 21 (80.8%) gene and cell therapies, fast track for 12 (46.2%), breakthrough therapy for 15 (57.7%), and regenerative medicine advanced therapy designation for 7 (26.9%).

EMA granted additional monitoring for 19 (79.2%) gene and cell therapies, conditional approval for 9 (37.5%), exceptional circumstances for 3 (12.5%), and accelerated assessment for 1 (4 .2%) therapy.

Figure 2. Orphan Cell and Gene Therapies Authorized by the FDA and the EMA (2009-2023)



Conclusions

A significant number of new cell and gene therapies have been approved by the FDA and EMA, mainly targeting rare cancers and genetic diseases. While the FDA often approved therapies first, the majority of cell and gene therapies were approved by only one agency.

Most therapies were authorized using regulatory designations and procedures available for the expedited development and regulatory review of new drugs. The EMA withdraw authorization to significant number of products.