Comparative Analysis of Gene Therapies Authorized by the U.S. Food and Drug Administration and the European Medicines Agency

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Objectives

This study compared the regulatory characteristics of gene therapies authorized by the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA).

Methods

Gene therapy regulatory data were extracted from the websites of the FDA and EMA as of December 31, 2025.

We descriptively compared authorization status, therapeutic class, regulatory designations and review pathways, authorization timing, and characteristics of approved indications.

Results

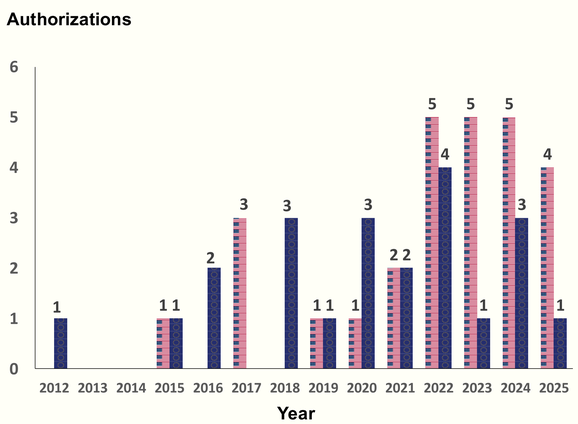
A total of 30 distinct gene therapies were authorized by at least one agency (Fig. 1).

The FDA authorized 27 (90.0%) therapies and the EMA 23 (76.7%) (including a therapy with EMA positive opinion and pending authorization from the European Commission). There were 20 (66.7%) gene therapies authorized by both agencies, 7 (23.3%) by FDA only, and 3 (10.0%) by EMA only.

The FDA authorized 21 (70.0%) gene therapies first, with a median EMA authorization gap of 310 (IQR, 334) days.

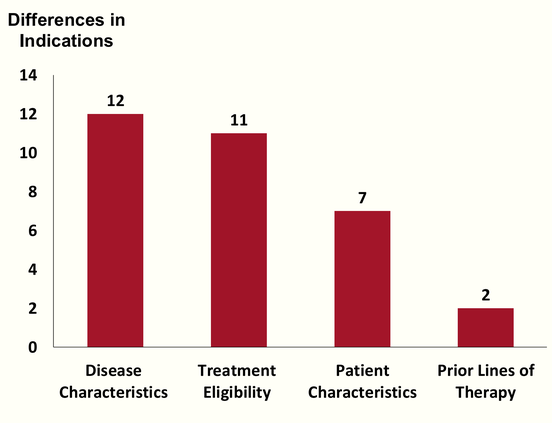
The most common therapeutic classes were antineoplastic & immunomodulating agents (14, 46.7%), blood and blood-forming organs (6, 20.0%), and alimentary tract & metabolism (3, 10.0%).

Figure 1. Gene Therapy Authorizations in the United States and European Union



Source: Compiled from the FDA and EMA websites, 2025.

Figure 2. Differences in Indications of Gene Therapies Authorized by FDA and EMA



Source: Compiled from the FDA and EMA websites, 2025.

Table 1. Orphan Designation and Expedited Regulatory Pathways for Gene Therapies

Gene Therapy	Orphan Designation		Expedited Review/Designation		Authorization Date	
	FDA	EMA	FDA	EMA	FDA	EMA
afamitresgene autoleucel	Y	N/A	Y	N/A	8/1/24	N/A
alipogene tiparvovec	N/A	N	N/A	Y	N/A	10/25/12
atidarsagene autotemcel	Y	Y	Y	Y	3/18/24	12/17/20
Autologous CD34+ cells transduced with an ADA-expressing retroviral vector	N/A	Y	N/A	Y	N/A	5/27/16
axicabtagene ciloleucel	Y	Y	Y	Y	10/18/17	8/23/18
beremagene geperpavec	Y	Y	Y	Y	5/13/23	9/23/24
betibeglogene autotemcel	Y	N	Y	Y	8/17/22	5/29/19
brexucabtagene autoleucel	Y	Y	Y	Y	7/24/20	12/14/20
ciltacabtagene autoleucel	Y	Y	Y	Y	2/28/22	5/25/22
delandistrogene moxeparvovec	Y	N/A	Y	N/A	6/22/23	N/A
eladocagene exuparvovec	Y	Y	Y	Y	11/13/24	7/18/22
elivaldogene autotemcel	Y	N	Y	Y	9/16/22	7/16/21
etranacogene dezaparvovec	Y	Y	Y	Y	11/22/22	2/20/23
etuvetidigene autotemcel	Y	Y	Y	Y	12/9/25	1/9/26
exagamglogene autotemcel	Y	Y	Y	Y	12/8/23	2/9/24
fidanacogene elaparvovec	Y	N	Y	Y	4/25/24	7/24/24
idecabtagene vicleucel	Y	Y	Y	Y	3/26/21	8/18/21
lisocabtagene maraleucel	Y	N	Y	Y	2/5/21	4/4/22
lovotibeglogene autotemcel	Y	N/A	Y	N/A	12/8/23	N/A
nadofaragene firadenovec	N	N/A	Y	N/A	12/16/22	N/A
nalotimagene carmaleucel	N/A	Y	N/A	Y	N/A	8/23/16
obecabtagene autoleucel	Y	Y	Y	Y	11/8/24	7/17/25
onasemnogene abeparvovec	Y	Y	Y	Y	5/24/19	5/18/20
prademagene zamikeracel	Y	N/A	Y	N/A	4/28/25	N/A
revakinagene taroretcel	Y	N/A	Y	N/A	3/5/25	N/A
talimogene laherparepvec	Y	None	Y	None	10/27/15	12/16/15
tisagenlecleucel	Y	Y	Y	Y	8/30/17	8/22/18
valoctocogene roxaparvovec	Y	Y	Y	Y	6/30/23	8/24/22
voretigene neparvovec	Y	Y	Y	Y	12/19/17	11/22/18
zopapogene imadenovec	Y	N/A	Y	N/A	8/14/25	N/A
Total	**26 (96.3%)**	**17 (73.9%)**	**27 (100.0%)**	**22 (95.7%)**	**27 (90.0%)**	**23 (76.7%)**

Orphan designation was granted to 26 (96.3%) therapies authorized by the FDA and to 17 (73.9%) therapies authorized by EMA. FDA regulatory pathways included priority review (24, 88.9%), breakthrough therapy designation (16, 59.3%), Regenerative Medicine Advanced Therapy designation (12, 44.4%), rolling review (12, 44.4%), fast track designation (9, 33.3%), and accelerated approval (8, 29.6%). EMA regulatory pathways included additional monitoring (21, 91.3%), priority medicines designation (17, 73.9%), and conditional marketing authorization (8, 34.8%).

One (4.3%) authorization expired and 3 (13.0%) were withdrawn from the market in the EU; there were 2 (7.4%) market discontinuations in the U.S. Among the 30 indications authorized by both agencies, 21 (70.0%) had at least one difference: 12 (40.0%) in disease characteristics, 11 (36.7%) in treatment eligibility, 7 (23.3%) in patient characteristics, and 2 (6.7%) in prior lines of therapy.

Conclusions

Most gene therapies were authorized by both agencies, with the FDA approving products earlier than the EMA by a median of less than one year.

Most therapies targeted rare diseases and received expedited development and regulatory review programs.

There were differences in gene therapy indications authorized by both agencies.

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