One US FDA / EMA Approval • 1/30 Approvals • 2/27/2026

GENE THERAPY APPROVALS & QPS MILESTONES

KEY

BIOANALYSIS • ADME • GENE THERAPY APPROVALS

QPS

2006
- CLIA Assay - Inclusion/ Exclusion Criteria
- Biomarkers - Primary End-point

2010
- LNP (Helper lipids, Ionizable lipids) *in vitro* Protein Binding
- LNP (Helper lipids, Ionizable lipids) *in vitro* Metabolic Stability
- LNP (Helper lipids, Ionizable lipids) Reaction Phenotyping

2010
- LNP (Helper lipids, Ionizable lipids) Metabolite Identification
- LNP (Helper lipids, Ionizable lipids) *in vitro* CYP Inhibition
- LNP (Helper lipids, Ionizable lipids) *in vitro* CYP Induction

2014
- mRNA PK/ Biodistribution *via* *RT-q*PCR
- Vector PK/ Biodistribution/ Shedding *via* qPCR

2016
- ADA for Gene Therapy Vectors (LNP and Viral Vectors)
- ADA from mRNA Expressed Proteins

2018
- CRISPR guide and CAS9 mRNA PK *via* RT-qPCR

2020
- LNP (Helper lipids, Ionizable lipids) PK/Bio distribution *via* LC-MS/MS

2022
- ddPCR for Insertion Copy Number/Biodistribution

2023
- dPCR and RT-dPCR

2024
- LNP *in vitro* CYP Inhibition
- LNP *in vitro* CYP Induction

2025
- Downstream Protein Quantitation *via* Immunocapture LC-MS/MS
- Immunophenotyping *via* Flow Cytometry
- Cellular Immunogenicity *via* Flow Cytometry
- Cellular Immunogenicity *via* ELISpot

GENE THERAPY

2012
***In vivo* Gene Insertion *via* AAV1**
Glybera®
(alipogene tiparvovec)

2015
***In vivo* Gene Insertion *via* HSV-1**
Imlygic®
(talimogene laherparepvec)

2016
***Ex vivo* Gene Insertion *via* Gammaretrovirus**
Strimvelis®
(autologous CD34+ enriched cell fraction)

2017
***Ex vivo* Gene Insertion *via* CAR-T**
Yescarta®
(axicabtagene ciloleucel)

***Ex vivo* Gene Insertion *via* CAR-T**
Kymriah®
(tisagenlecleucel)

***In vivo* Gene Insertion *via* AAV2**
Luxturna®
(voretigene neparvovec)

2019
***In vivo* Gene Insertion *via* AAV9**
Zolgensma®
(onasemnogene abeparvovec)

2020
***Ex vivo* Gene Insertion *via* LVV**
Libmeldy®/Lenmeldy™
(atidarsagene autotemcel)

2021
***Ex vivo* Gene Insertion *via* CAR-T**
Abecma®
(idecabtagene vicleucel)

***Ex vivo* Gene Insertion *via* CAR-T**
Tecartus®
(brexucabtagene autoleucel)

2022
***Ex vivo* Gene Insertion *via* CAR-T**
Carvykti®
(ciltacabtagene autoleucel)

2022
***Ex vivo* Gene Insertion *via* CAR-T**
Breyanzi®
(lisocabtagene maraleucel)

***In vivo* Gene Insertion *via* AAV5**
Roctavian™
(valoctocogene roxaparvovec)

***In vivo* Gene Insertion *via* AAV2**
Upstaza™/Kebilidi™
(eladocagene exuparvovec)

***Ex vivo* Gene Insertion *via* LVV**
Zynteglo®
(betibeglogene autotemcel)

2022
***Ex vivo* Gene Insertion *via* LVV**
Skysona™
(elivaldogene autotemcel)

***In vivo* Gene Insertion *via* AAV5**
Hemgenix®
(etranacogene dezaparvovec)

***In vivo* Gene Insertion *via* AdV5**
Adstiladrin®
(nadofaragene firadenovec)

2023
***In vivo* Gene Insertion *via* HSV-1**
Vyjuek™
(beremagene geperpavec)

***In vivo* Gene Insertion *via* AAVrh74**
Elevidys™
(delandistrogene moxeparvovec)

***Ex vivo* Gene Editing via CRISPR/Cas9**
Casgevy®
(exagamglogene autotemcel)

***Ex vivo* Gene Editing *via* LVV**
Lyfgenia™
(lovotibeglogene autotemcel)

2024
***In vivo* Gene Insertion *via* AAVRh74var**
Beqvez™
(fidanacogene elaparvovec)

***Ex vivo* Gene Insertion *via* LVV**
Tecelra®
(afamitresgene autoleucel)

***Ex vivo* Gene Editing *via* CAR-T**
Aucatzyl®
(obecabtagene autoleuce)

2025
***Ex vivo* Gene Editing *via* rhCNTF**
Encelto™
(revakinagene taroretcel)

***Ex vivo* Gene Editing *via* RVV**
Zevaskyn®
(prademagene zamikeracel)

***In vivo* Gene Insertion *via* AdV**
Papzimeos™
(zopapogene imadenovec)

***In vivo* Gene Insertion *via* AAV9**
Itvisma™
(onasemnogene abeparvovec-brve)

2025
***Ex vivo* Gene Insertion *via* LVV**
Waskyra™
(etuvetidigene autotemcel)