HERMS WHITE PAPER | NOVEMBER 2026

Gene Therapy 2026 From Breakthrough to Gulf Patient Access

Approvals, technology shifts, regulatory change and the trials to watch, with a readiness framework for GCC health systems and developers.

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EX EC U TIV E SU M M A RY

2026 is the year gene therapy became a system, not an exception

Approvals, in-body gene editing and new payment models are moving gene therapy from isolated breakthroughs to a repeatable treatment category. GCC health systems that prepare now will deliver these therapies to patients years earlier.

2,257

gene therapies in development, Q3 20261

62

gene therapies in Phase III1

11

gene therapies filed, awaiting approval1

49

gene therapies approved worldwide1

Five findings

Approvals are broadening. In 2026 the US FDA approved new gene therapies for immune

deficiency, genetic hearing loss, glycogen storage disease, Sanfilippo syndrome and melanoma, and extended Casgevy to children with sickle cell disease aged two and older.2–6

Editing has moved inside the body. The first Phase III trial of an in vivo CRISPR therapy succeeded, and prime editing was used directly in a patient for the first time.9,11

Regulators are adapting to personalised therapies. FDA draft guidance allows one licence to cover multiple patient-specific gene-editing variants without new clinical trials for each.8

Payment is shifting to outcomes. The US CMS is extending outcomes-based agreements for cell and gene therapies beyond sickle cell disease.8

The Gulf is already a gene therapy region. Bahrain, Saudi Arabia and the UAE have approved

Casgevy, and Abu Dhabi administered it to a patient for the first time in January 2026.20–23

HERMS view. The limiting factor for Gulf patients is no longer the existence of therapies. It is readiness: regulatory pathways, qualified treatment centres, cell and vector logistics, long-term follow-up and sustainable payment. Each month of delay carries clinical cost for patients with progressive genetic disease.

SEC TION 1

The 2026 approval wave

Gene therapy approvals in 2026 have spread across disease areas, delivery routes and age groups. Several are first-ever treatments for their condition, and one is the first dual-vector AAV therapy.

Product	Indication	Technology	US approval
Kresladi (marnetegragene autotemcel)	Severe leukocyte adhesion deficiency type I in children; first gene therapy for the condition2	Ex vivo, modified stem cells	26 Mar 2026
Otarmeni (lunsotogene parvec), Regeneron	Severe-to-profound hearing loss caused by biallelic OTOF variants; first dual-AAV vector gene therapy3	AAV1, injected into the cochlea	23 Apr 2026

Casgevy

(exagamglogene autotemcel), label expansion

Sickle cell disease in children aged 2 years and older4

Ex vivo CRISPR

1 Jul 2026

Genglycos

(pariglasgene brecaparvovec)

Glycogen storage disease type Ia, aged 8 and older; accelerated approval to reduce daily cornstarch use5

AAV, liver-directed

19 Aug 2026

Fayuvi (rebisufligene

etisparvovec)

Sanfilippo syndrome type A (MPS IIIA) in children; first approved treatment6

AAV

17 Sep 2026

Tudriqev

(vusolimogene oderparepvec), Replimune

Melanoma1

Oncolytic gene therapy

Q3 2026

Plasmid DNA

immunotherapy

Recurrent respiratory papillomatosis in adults; first

plasmid DNA-based immunotherapy7

Plasmid DNA

30 Oct 2026

Under review: decisions due in 2027

Therapy

Indication

Status

Lonvo-z (lonvoguran

ziclumeran), Intellia

Hereditary angioedema

First in vivo CRISPR therapy filed; FDA decision

due by 10 March 202710

AMT-130, uniQure

Huntington's disease

Accepted with priority review under accelerated

approval; target date 1 May 2027, advisory

committee planned15

Pattern. Most 2026 approvals target rare, severe, childhood-onset genetic diseases with no prior treatment. The age of eligible patients is falling, which raises the importance of early diagnosis and newborn or premarital genetic screening.

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SEC TION 2

The pipeline: where development is concentrated

The gene therapy pipeline reached 2,257 programmes in Q3 2026, up from 2,128 a year earlier. Counts increased at every stage except pre-registration between Q2 and Q3 2026.1

Gene therapy pipeline by development stage, Q3 2026

Preclinical

1,355

Phase I

437

Phase II

392

Phase III

62

Pre-registration

11

Source: ASGCT / Citeline Gene, Cell & RNA Therapy Landscape Report, Q3 2026.1

Gene therapy pipeline by therapeutic area (programmes), Q3 2026

Anticancer

1,142

Rare disease

1,101

Neurological

270

Sensory

204

Immunological

197

Therapeutic areas are not mutually exclusive. Source: ASGCT Q3 2026.1

Oncology leads

Oncology accounts for 54% of rare-disease gene therapies in the pipeline and 65% of those in clinical trials. The most common targets are myeloma, non-Hodgkin's lymphoma and B-cell lymphoma.1

Beyond rare disease

Among gene therapies for common

conditions, solid tumours (379 programmes) and autoimmune disease (118) are the most frequent targets.1 Lipid-lowering in vivo editing is also advancing.14

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SEC TION 3

Technology shift: editing moves inside the body

First-generation gene-edited therapies, such as Casgevy, modify a patient's stem cells outside the body and need conditioning chemotherapy and transplant-level care. The 2026 clinical data show editing delivered directly into the body, typically to the liver, as a single infusion.

Approach

2026 milestone

Why it matters

In vivo CRISPR

Lonvo-z met its primary and secondary endpoints in an 80-patient, randomised, double-blind Phase III trial in hereditary angioedema.9

First Phase III proof that a one-time, in-body gene edit can replace chronic therapy.

Base editing

Beam had dosed 38 patients with in vivo base editor BEAM-302 for alpha-1 antitrypsin deficiency by August 2026.13

Changes single DNA letters without cutting both strands.

Prime editing

First known use of prime editing directly inside a person, in Prime Medicine's Phase 1/2 trial of PM577a for Wilson disease.11,12

Can correct a wider range of mutations with

high precision.

Common-disease

editing

CRISPR Therapeutics' CTX310 (ANGPTL3)

produced deep, durable reductions in

triglycerides and LDL cholesterol.14

Extends gene editing from rare to high-

prevalence cardiometabolic disease.

Multi-vector AAV

Otarmeni became the first dual-AAV vector

gene therapy approved.3

Delivers genes too large for a single AAV

vector.

Safety remains the central constraint

Reviews of AAV gene therapy describe a clear link between systemic dose and toxicity, including immune responses against the capsid, liver burden and complement activation that can lead to thrombotic microangiopathy.19 These risks shape dose selection, immunosuppression protocols, patient monitoring and the clinical capability required at treatment centres.

Implication for providers. In vivo therapies may reduce the need for transplant-level infrastructure, but they still require specialist infusion, acute safety management and multi-year follow-up.

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SEC TION 4

Regulation and payment are being rebuilt

FDA: a framework for individualised gene editing

On 27 October 2026 the FDA issued draft guidance, Individualized Human Genome Editing Products: Considerations for BLA Submission and Postapproval Management.8

One licence application can cover multiple patient-specific variants of a gene-editing product.

An **Individualized Feature Space** sets the boundaries within which a product can vary between patients.

New variants inside those boundaries can be added using laboratory data, without new clinical trials.

The guidance also covers manufacturing, safety testing for unintended genetic changes, naming and labelling.

On 2 November 2026 the FDA also published guidance on potency assurance for cell and gene therapy products, a core manufacturing quality requirement.7

CMS: outcomes-based access beyond sickle cell disease

CMS announced it will broaden its Cell and Gene Therapy Access Model beyond sickle cell disease. CMS negotiates outcomes-based agreements with manufacturers on behalf of state Medicaid programmes. Its first evaluation found that pooled negotiation reduced state administrative burden and strengthened negotiating leverage.8

What GCC regulators can adopt

Reliance on reference-agency assessments for faster review.

Platform and variant-based approaches for personalised therapies.

Clear potency and comparability expectations for imported products.

What GCC payers can adopt

Outcomes-based agreements linked to durable response.

Pooled or joint negotiation across emirates or GCC states.

Instalment payments aligned with multi-year follow-up.

SEC TION 5

Trials to watch

Therapy and sponsor

Indication

Stage

Latest update

AMT-130, uniQure

Huntington's disease

Under FDA

review

Priority review, target 1 May 202715

Lonvo-z, Intellia

Hereditary angioedema

Under FDA

review

Positive Phase III HAELO; decision by

10 Mar 20279,10

OCU410, Ocugen

Geographic atrophy (dry

AMD)

Phase III

(ArMaDa3)

First patient dosed; Phase 2 showed

31% less lesion growth vs control16

Elevidys

(delandistrogene

moxeparvovec)

Duchenne muscular

dystrophy

Phase III

EMBARK

Three-year follow-up published17

PM577a, Prime Medicine

Wilson disease

Phase 1/2

First in vivo prime-editing patient

dosed12

BEAM-302, Beam

Alpha-1 antitrypsin

deficiency

Clinical

38 patients dosed by Aug 202613

CTX310, CRISPR

Therapeutics

Severe

hypertriglyceridaemia,

raised LDL

Phase 1a

Durable lipid reductions; RMAT

designations14,24

Kamau Therapeutics

Sickle cell disease

Early clinical

First data from revamped gene-editing

therapy at ASH 202618

Gulf relevance. Sickle cell disease, inherited metabolic and liver disorders, hereditary hearing loss and retinal disease are all represented in this pipeline. These are conditions with meaningful prevalence in Gulf and Arab populations.

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SEC TION 6

The GCC picture: early adopters with high need

The Gulf has already shown it can move quickly. Bahrain became the second country in the world, and the first in the Middle East, to approve Casgevy in December 2023.21 Saudi Arabia approved it in January 2024 as the first medicine evaluated through the SFDA's Breakthrough Medicines Program.20 Abu Dhabi's Department of Health introduced Casgevy in March 2025, and the first UAE patient was treated at Yas Clinic in January 2026.22,23 Casgevy has since been approved in the UAE and Bahrain for children aged two and older.24

The need is substantial

In Saudi Arabia, reported sickle cell trait prevalence ranges from 2% to 27% by region, with up to 2.6% having sickle cell disease in some areas.25

Saudi Arabia's national premarital screening programme identified 4.5% of those examined as carriers or cases of sickle cell disease, and 1.8% for beta-thalassaemia, over its first six years.26

Regulatory foundations in place

Saudi Arabia offers standard, abridged and verification pathways that can apply to advanced therapies, depending on reference-agency approvals.27

In the UAE, the Emirates Drug Establishment's federal remit covers stem cells and genetically modified organisms, alongside medicines and devices.28

The remaining gaps

Gap

Effect on patients

Qualified treatment centres

Few sites can deliver ex vivo editing, so patients may wait or travel.

Cell and vector logistics

Cryogenic shipping, import permits and chain of identity add time and risk.

Diagnosis and referral

Eligible patients without confirmed genotype or referral are never assessed.

Payment models

One-time high-cost therapies strain annual budgets without outcomes-based or instalment models.

Long-term follow-up

Multi-year safety monitoring needs registries and pharmacovigilance designed

for delayed events.

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SEC TION 7

The HERMS Gene Therapy Readiness Framework

HERMS assesses market and institutional readiness for gene therapy across eight pillars. Each pillar must be in place before a therapy can reach patients reliably and safely.

1. Regulatory pathway

Route selection (standard, reliance, breakthrough), dossier alignment with reference approvals, lifecycle variation plan.

2. Treatment centre qualification

Accredited sites, trained teams, apheresis and infusion capability, acute toxicity management.

3. Logistics and chain of identity

Cryogenic shipping, import permits, customs, end-to-end traceability from patient cells to infusion.

4. Patient identification

Screening, genetic confirmation, referral pathways and eligibility criteria.

5. Pharmacovigilance

Risk management plans, local QPPV, signal detection for delayed and rare adverse events.

6. Long-term follow-up

Registries and data systems for multi-year efficacy and safety monitoring.

7. Value and payment

Health economic evidence, outcomes-based agreements, instalment and pooled-purchase models.

8. Evidence governance

Source-traceable records, audit trails and accountable human sign-off for every consequential decision.

Recommendations

For developers

Plan GCC filings alongside FDA and EMA submissions, not after them.

Use reliance pathways where eligible.

Qualify at least one regional treatment centre before launch.

Propose outcomes-based payment from the start.

For authorities and payers

Publish clear advanced-therapy guidance and timelines.

Pilot outcomes-based agreements for haemoglobinopathies.

Link premarital and newborn screening to referral pathways.

Build national gene therapy registries.

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SEC TION 8

How HERMS supports gene therapy access

HERMS works with developers, health authorities and providers to shorten the time from global approval to Gulf patient access.

Regulatory strategy: pathway assessment, dossier preparation and submission management across GCC authorities.

Readiness assessments: structured evaluation against the eight-pillar framework for markets and treatment centres.

Market access: value dossiers, budget-impact analysis and outcomes-based agreement design.

Pharmacovigilance: local QPPV services, risk management plans and long-term follow-up systems.

Governed evidence: source-traceable intelligence with human accountability for every decision.

Request a gene therapy readiness assessment. info@hermsglobal.com · +971 55 600 8944 ·

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Related HERMS resources

Cell and gene therapy readiness in the GCC

GCC pharmaceutical pricing and market access strategy

GCC pharmacovigilance obligations for marketing authorisation holders

UAE pharmaceutical product registration

Contact HERMS

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