XERAYA CAPITAL
Life Venture

2026

GENE THERAPY & GENE EDITING

THE NEXT FRONTIER OF PRECISION MEDICINE

April 2026

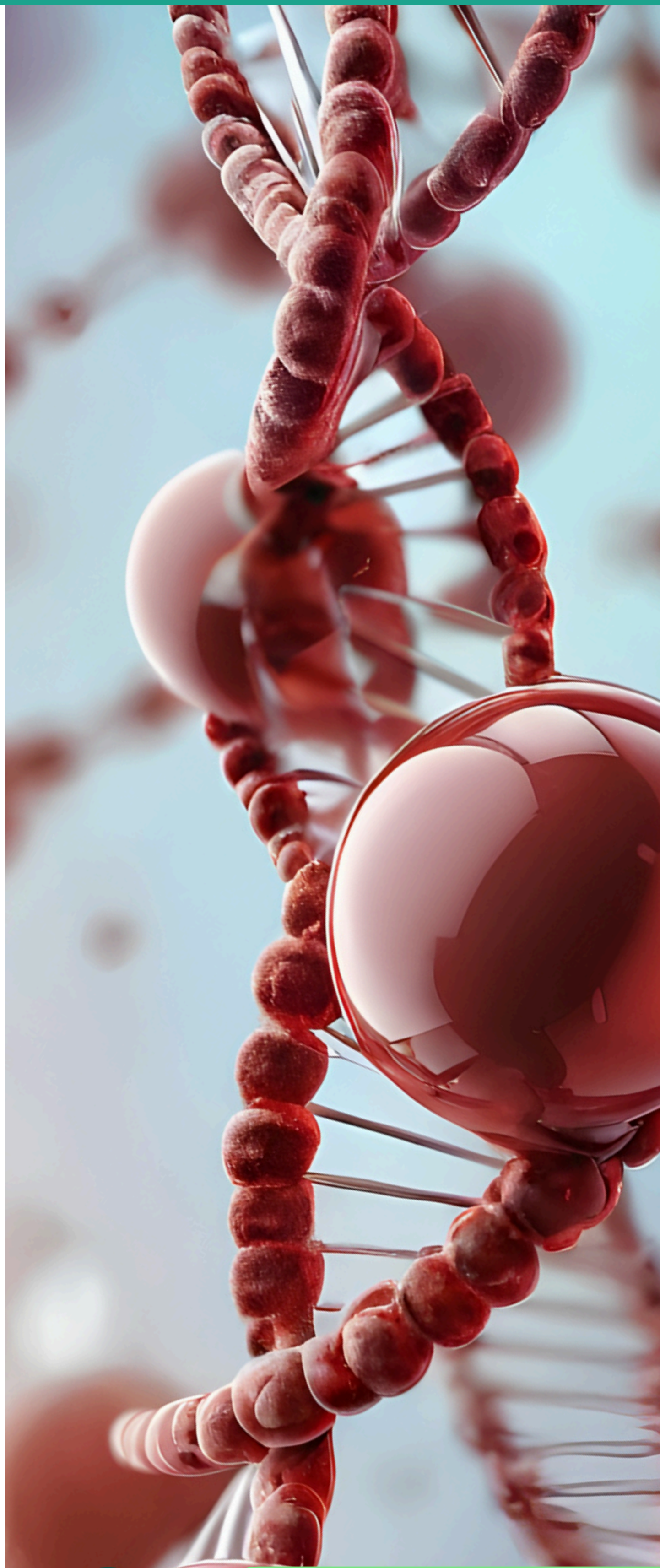
Executive Summary

Historically, the structural paradigm of modern healthcare has been inherently constrained: clinical medicine has overwhelmingly focused on treating symptoms such as managing pain, lowering fevers, or replacing failing organs. This reactive model has frequently fallen short, particularly when confronting complex, incurable genetic anomalies or novel pathogens.

Gene therapy and gene editing aim to systematically address disease at its very source, the foundational genetic instructions coded within our cells. What was recently viewed as experimental science fiction has now matured into a robust, high-growth scientific and commercial reality.

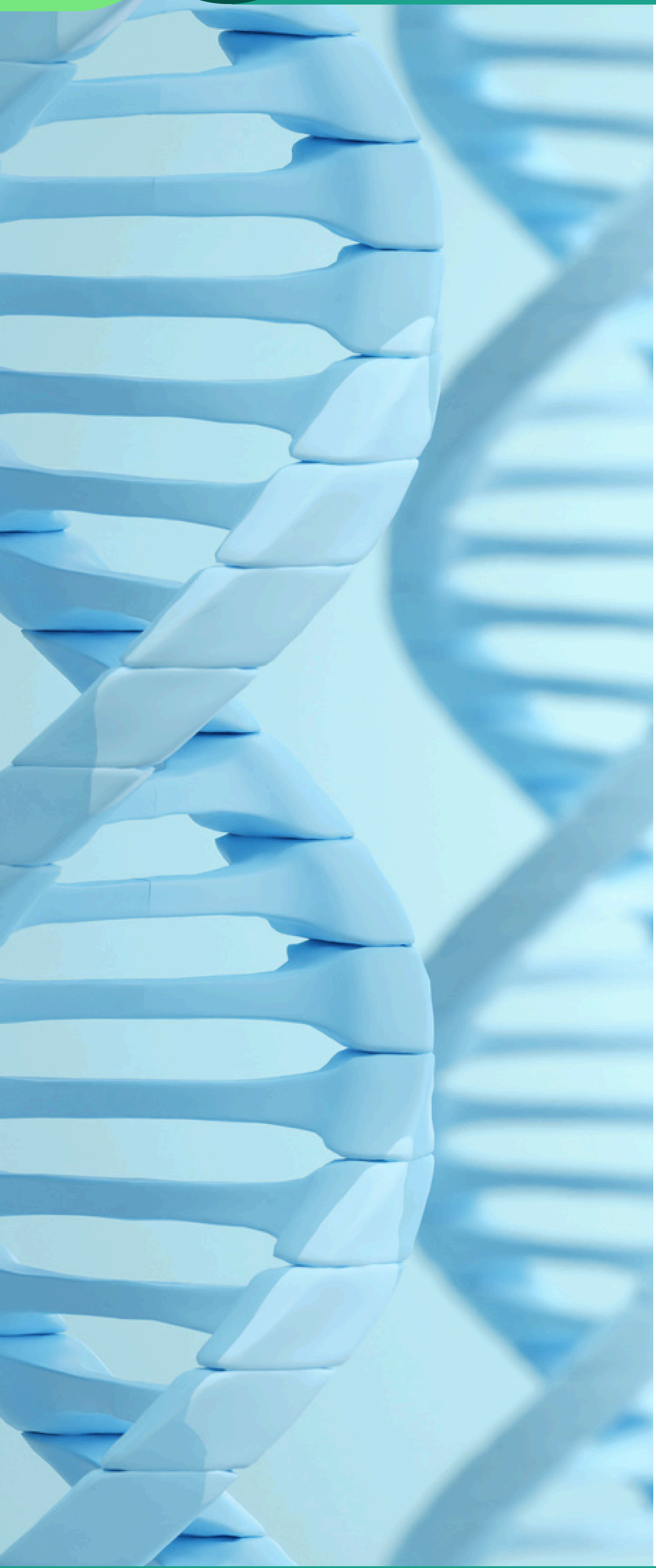
This allows us to rewrite the human biological code, one letter at a time, we are investing in the definitive future of medicine.

Market Sizing & Growth Dynamics



The commercial velocity of the cell and gene therapy (CGT) sector continues to outpace broader pharmaceutical benchmarks. Based on updated 2026 market intelligence from Precedence Research, the global gene therapy market reached an estimated valuation of **USD 11.07 billion** in 2025. Driven by aggressive clinical pipelines and widespread regulatory momentum, the Total Addressable Market (TAM) is projected to scale at a formidable **19.60% Compound Annual Growth Rate (CAGR),** targeting a valuation exceeding **USD 55 billion** by 2034.

This explosive TAM expansion is deeply rooted in physiological realities. At its core, human DNA functions as an intricate, 3 billion-letter instruction manual. These sequences dictate every biological function, from how our heart beats and lungs breathe to our fundamental physical traits. However, an infinitesimal error, a single misplaced or missing letter, can synthesize a dysfunctional protein, triggering a lifetime of severe, debilitating illness.



For instance, in sickle cell disease, a singular change in the DNA code alters the structure of hemoglobin, causing blood cells to become stiff and crescent-shaped, which leads to severe pain, infections, and systemic organ damage. Similarly, cystic fibrosis is driven by a faulty gene that disrupts the movement of salt and water in and out of cells, producing thick mucus that severely clogs the lungs and digestive system. Furthermore, missing or faulty genetic instructions can lead to conditions like spinal muscular atrophy, a disease that weakens muscles and can be fatal in infancy.

Because conventional treatments have historically been limited to symptom mitigation when a body fails to produce a crucial protein, a revolutionary technological leap was required to permanently correct these genetic typos. The commercialization of these breakthroughs is no longer theoretical. For example, Casgevy, the world's first CRISPR-based therapeutic, saw a nearly three-fold global increase in patient initiations in 2025 for sickle cell disease and beta thalassemia, validating the massive commercial appetite for curative therapies.

Can All Diseases Be Treated with Gene Therapy?

Not every disease can be fixed with gene therapy or gene editing. The most promising targets are conditions caused by mutations in a single gene. If a single faulty instruction causes the problem, correcting it can restore functionality of the mutated protein. Blood disorders are particularly suitable because blood stem cells can be removed, modified, and returned. Certain inherited eye disorders are also attractive targets because the eye is relatively self-contained and requires minimal treatment. Gene therapy is also often used for hereditary diseases or rare diseases.

On the other hand, complex conditions, such as diabetes, heart disease, or Alzheimer’s disease, involve many genes interacting with environmental factors. Correcting one genetic change would not be enough. Fixing one typo is far easier than rewriting an entire chapter. However, with advances in gene editing, treating these complex conditions with gene therapy may not be far off.

The Technological & Macro Catalyst

The current market tailwinds are catalyzed by the concurrent maturation of two distinct, yet complementary, genetic modalities:

GENE EDITING (THE "CODE REWRITE"):

If gene therapy acts as an addendum, gene editing rewrites the original, corrupted sentence. The primary driver of this revolution is the CRISPR/Cas9 platform. Utilizing a highly specific piece of RNA as a molecular GPS, the system navigates Cas9 enzymes, acting as biological scissors, to exact genomic coordinates. Upon cutting the targeted DNA sequence, the cell's natural repair pathways are hijacked to either disable a faulty gene or precisely correct the initial error.

GENE THERAPY

Gene therapy operates much like inserting a corrected page into a corrupted instruction manual. Rather than erasing the underlying mutation, this modality introduces a healthy, functional copy of the target gene, enabling the cell to accurately synthesize the required protein. To deliver this payload into billions of cells, developers utilize highly modified, deactivated viruses as microscopic delivery vehicles. These viral vectors are inherently adept at penetrating cellular walls, facilitating long-lasting and potentially permanent therapeutic expression. This process provides a working backup, conceptually identical to deploying a software update that overrides a systemic bug without deleting the legacy code. A leading commercial proxy is Zolgensma, an approved gene therapy that delivers a functioning gene to infants suffering from spinal muscular atrophy, a devastating and previously fatal neuromuscular disease.

These technologies are profoundly restructuring care protocols for diseases like sickle cell, where a single DNA error misshapes red blood cells, causing systemic organ damage, and cystic fibrosis, where faulty genes disrupt cellular fluid regulation.

Because these illnesses stem exclusively from biological typos rather than lifestyle factors or infections, correcting the code fundamentally eradicates the disease state.

The Delivery Pivot: Ex Vivo vs. In Vivo

Macro commercial viability is heavily dictated by delivery mechanisms. Current commercial frontrunners heavily leverage an ex vivo (“outside the body”) approach, extracting a patient's cells (such as blood stem cells), editing them in highly controlled laboratory environments, and subsequently re-infusing them. This is particularly viable for blood disorders, as stem cells can be reliably collected and transplanted. Ex vivo methods offer unparalleled safety controls, as modifications are meticulously verified before re-entry.

However, scaling these treatments requires navigating immense clinical bottlenecks. Consequently, significant venture capital is now flowing toward in vivo therapies. By delivering genetic modifications directly into the patient’s body via targeted injections or infusions, in vivo platforms bypass complex cellular transplants entirely, though they demand extraordinary precision to ensure only target tissues are impacted.

Regional Competitive Advantage: The Malaysian Imperative

Geographical capital deployment requires strategic alignment with macroeconomic policy and regulatory clarity. While Singapore currently maintains a strong foothold in ASEAN as a center for advanced biomanufacturing and multinational biotech presence, Malaysia has rapidly positioned itself as a high-growth, highly attractive platform for gene therapy investment.

Malaysia's regional value proposition is anchored in several distinct strategic enablers:

1. REGULATORY MATURITY

The National Pharmaceutical Regulatory Agency (NPRA) has successfully cultivated a structured, dynamic regulatory pathway specifically designed for Cell and Gene Therapy Products (CGTPs). By aligning with international standards, the NPRA has heavily derisked clinical development. The landmark approval of Zolgensma critically validated Malaysia's capability to assess, authorize, and integrate complex viral vector-based therapeutics.

2. POLICY SYNERGIES & THE 13MP

Under the newly launched 13th Malaysia Plan (2026–2030), the bioeconomy has been classified as a core national priority. Initiatives driven by the Malaysian Bioeconomy Development Corporation actively foster public-private collaborations, establish regulatory sandboxes, and heavily reduce bureaucratic fragmentation.

3. GROWTH HEADROOM

Over the next three to five years, Malaysia possesses the requisite infrastructure and competitive operational costs to dramatically accelerate clinical trials, scale CAR-T capabilities, and attract high-value manufacturing partnerships across the ASEAN bloc.

Conclusion

The biotechnology sector has crossed a critical threshold. We are no longer limited to merely managing biological decay; we possess the tools to correct life's fundamental code. Today, infants suffering from rare, historically lethal genetic disorders can receive personalized, curative gene therapies in a matter of months.

This is not a transient technological trend; it is a permanent evolution in medical science. Gene therapy will tangibly improve millions of lives globally. Allocating strategic capital into clinical pioneers, delivery IP, and the rapidly maturing manufacturing ecosystems of Malaysia and the wider ASEAN region presents a rare opportunity to capture immense financial upside while backing the definitive future of human health.

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