Rewriting Disease: Gene Therapy and Gene Editing in Today's Pharmacy

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Disclosure Statement

Drs. Nicola Carter and Sigrid Roberts have no relevant financial relationship(s) with ineligible companies to disclose.

Learning Objectives

- Describe the basic principles of gene editing versus gene therapy
- Discuss the promises and challenges of gene editing for pharmaceutical treatment strategies
- List currently available treatments based on gene editing

Pre-Test Questions

1. **Which of the following best distinguishes a "gene editing" therapeutic approach from traditional gene therapy? Gene editing:**
   A. requires periodic re-administration to maintain the therapeutic effect.
   B. uses a nuclease to permanently modify an endogenous DNA sequence.
   C. relies exclusively on viral vectors to deliver content to the nucleus.
   D. introduces a functional gene copy without altering the host genome.

Pre-Test Questions

2. **Which of the following represents a significant "promise" of gene editing in the context of pharmaceutical treatment strategies?**

   A. Transitioning from chronic symptom management to a "one-and-done" curative approach
   B. Immediate reduction in the Total Cost of Care (TCC) for the first year of treatment
   C. May be delivered via community-based speciality pharmacies with standard cold-chain storage
   D. Minimizing the requirement for long-term patient monitoring

Pre-Test Questions

3. **As of 2026, which of the following is an FDA-approved gene editing therapy utilized for the treatment of Sickle Cell Disease and Beta Thalassemia?**

   A. Voretigene neparvovec (Luxturna)
   B. Onasemnogene abeparvovec (Zolgensma)
   C. Exagamglogene autotemcel (Casgevy)
   D. Tisagenlecleucel (Kymriah)

Central Molecular Biology Dogma

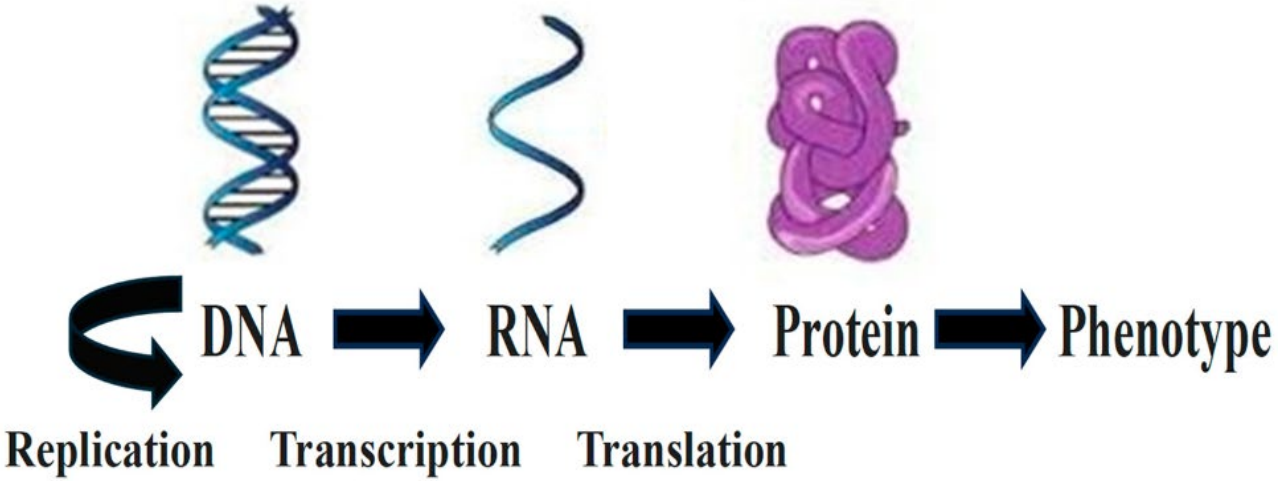


Image source: Haseltine, W. A., & Patarca, R. (2024). The RNA Revolution in the Central Molecular Biology Dogma Evolution. *International Journal of Molecular Sciences*, *25*(23), 12695. https://doi.org/10.3390/ijms252312695

Gene Therapy *vs.* Gene Editing

- Functional gene copy delivered to and added to patient DNA to compensate for faulty/non-functioning gene
- Other iteration involves gene suppression

- DNA is cut, edited, or replaced to rewrite the patient's DNA sequence

Image source: https://cdn.drugdiscoverynews.com/assets/image/4668/gene-therapy-vs-gene-editing-l.jpg

Gene Therapy

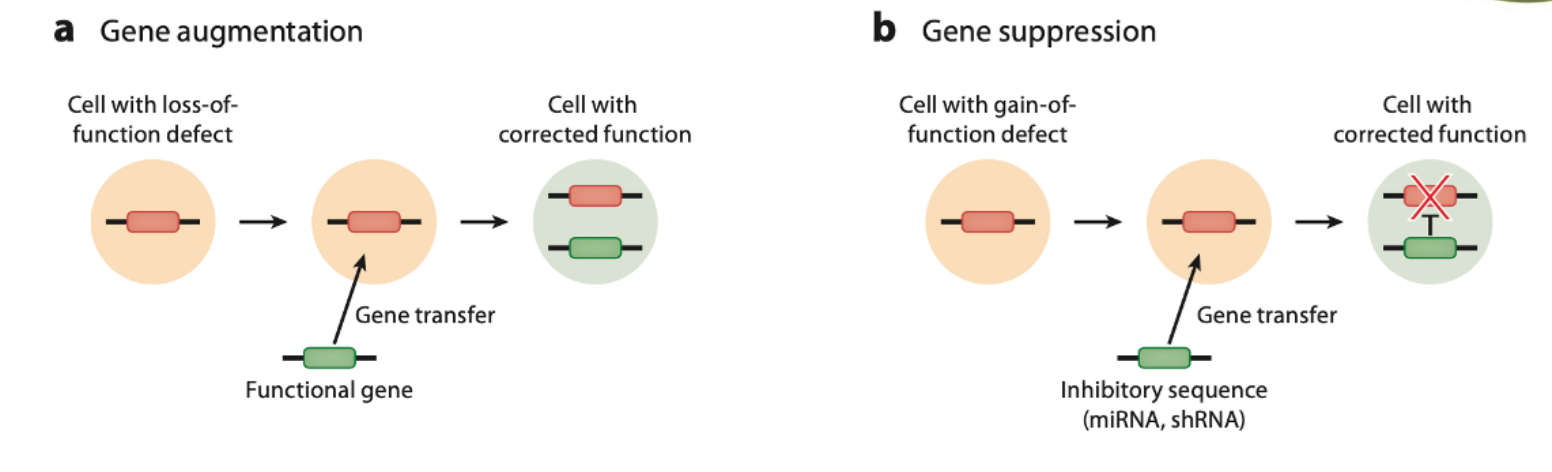


Image source: Anguela, X. M., & High, K. A. (2019). Entering the Modern Era of Gene Therapy. Annual review of medicine, 70, 273–288. https://doi.org/10.1146/annurev-med-012017-043332

Gene Editing

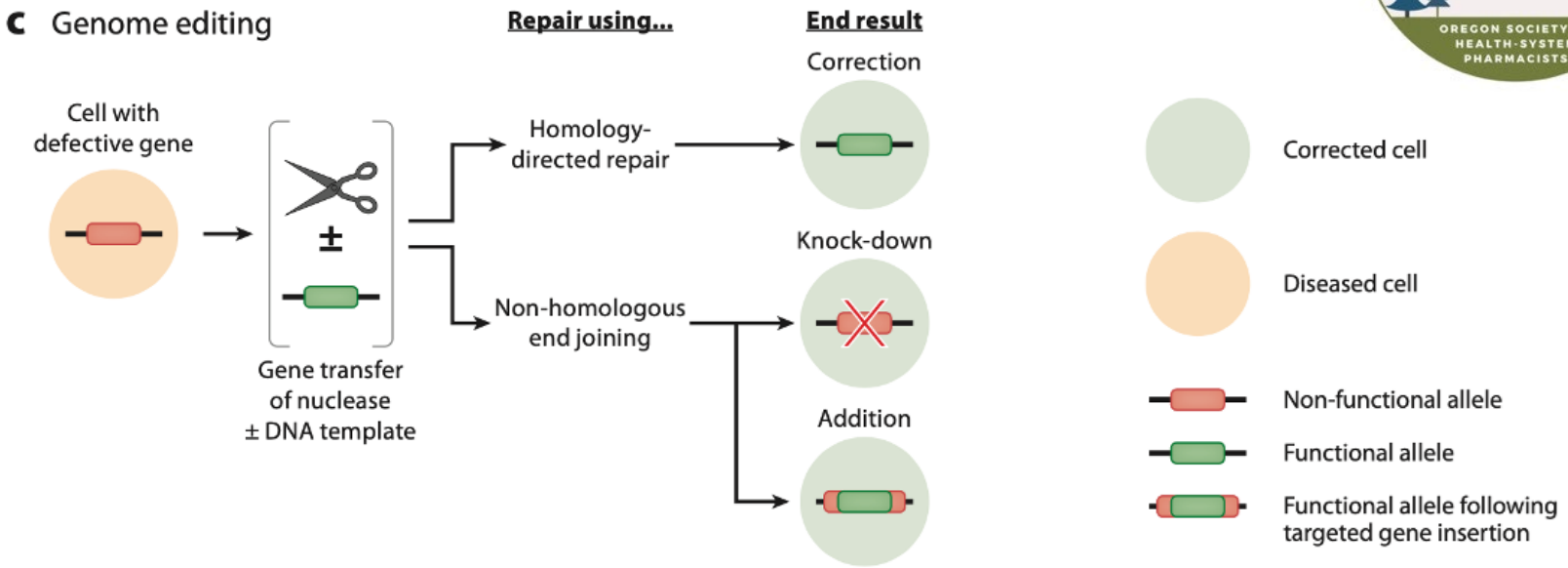


Image source: Anguela, X. M., & High, K. A. (2019). Entering the Modern Era of Gene Therapy. Annual review of medicine, 70, 273–288. https://doi.org/10.1146/annurev-med-012017-043332

Cell Therapy

"Living cells" as drugs

- Activated cells
- Genetically engineered cells

Example: T-cell transfer in cancer therapy
CAR T-cell therapy: Chimeric Antigen Receptor (CAR) T-Cell Therapy

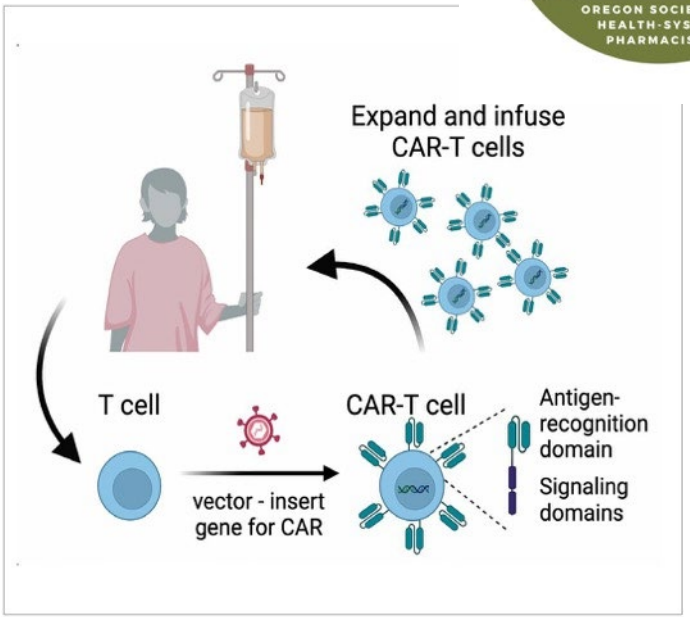
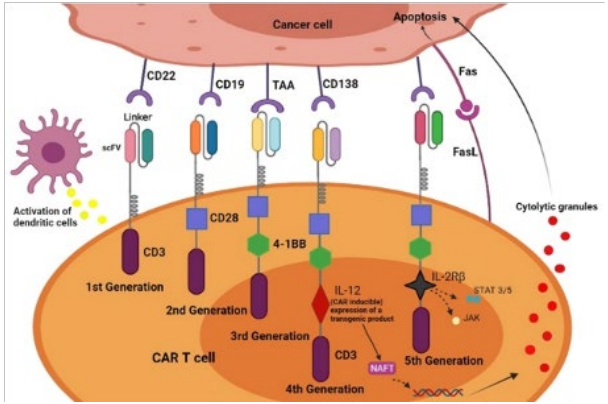


Image source: Magnus Essland. CAR T Cells Engineered to Prevent Antigen Escape: Elicera Therapeutics describes how CAR T cells armed with neutrophil-activating protein can improve cancer therapy by inducing bystander immunity. Genetic Engineering & Biotechnology NewsVolume 44, Issue 10, 1 October 2024, Pages 42-43

Cell Therapy

Chimeric Antigen Receptor (CAR) T-cell therapy



Source: Mehrabadi et al. Therapeutic potential of CAR T cell in malignancies: A scoping review. Biomed Pharmacother. 2022 Feb;146:112512. doi: 10.1016/j.biopha.2021.112512.

First generation: Extracellular targeting domain linked via a hinge and transmembrane spacer to intracellular signaling domains

Second/Third generation: One or two co-stimulatory domains added to enhance T cell activation and proliferation

Fourth generation (TRUCKs): T cells redirected for universal cytokine-mediated killing via added cytokine secretion modules

Fifth (next) generation: Enables JAK-STAT signaling, aiming to overcome T-cell exhaustion and improve efficacy (early clinical trials)

Currently 7 FDA approved CAR T-cell therapies

Gene Delivery Methods

Ideal: Precise, safe, and efficient transfer of DNA into the nucleus of affected cells, and provide long-lasting results

Viral delivery

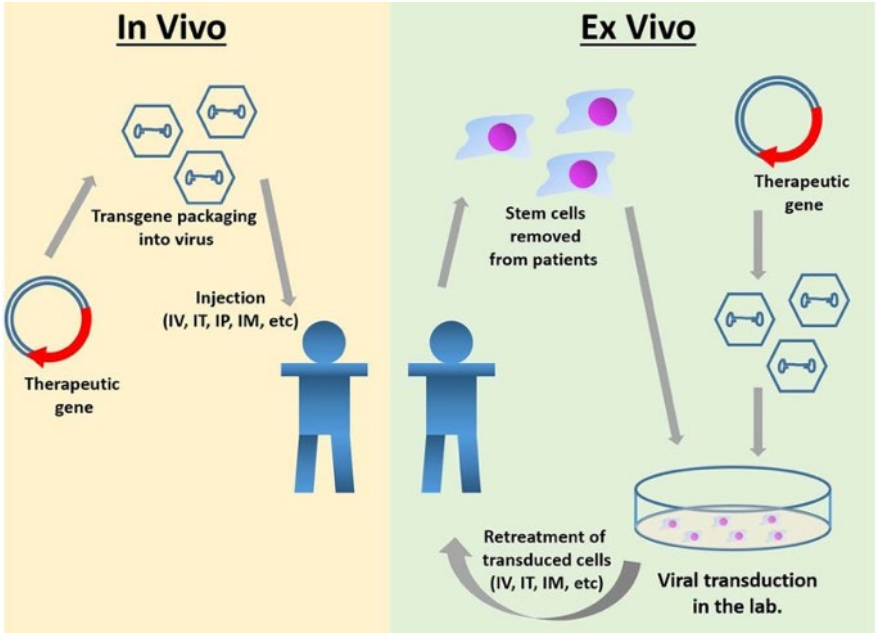
- Gene addition, shRNA, gene editing
- Highly efficient, long-lasting
- Genetically engineered to not replicate once introduced into patient
- Can elicit an immune response in some individuals
- Lentivirus (genome integration), Adeno-Associated Virus (episomal)

Non-Viral Delivery

- siRNAs, gene editing
- Variable efficiency, transient, re-dosable
- Potential for dose-limiting toxicity
- Methods:
  - Physical/Mechanical: Electroporation or direct Injection of naked DNA/RNA
  - Lipid Nanoparticles (LNP)
  - Synthetic nanoparticles

Source: Anguela, X. M., & High, K. A. (2019). Entering the Modern Era of Gene Therapy. *Annual review of medicine*, *70*, 273–288.

Gene Therapy Delivery: Ex Vivo *vs.* In Vivo



Major cell types:
Hematopoietic stem cells
Immune cells (CAR T-cells)

Image source: :https://www.researchgate.net/profile/Shunji-Tomatsu/publication/322970469/figure/fig1/AS:593727915581440@1518567174798/Strategies-of-in-vivo-gene-therapy-and-ex-vivo-gene-therapy-In-vivo-gene-therapy-on-the.png

One-and-Done Therapeutic Paradigm

Curative Intent: Unlike chronic "maintenance" drugs (pills/biologics), gene therapy/editing aims to address the **genetic cause** in a single intervention

In Vivo: Stable transgene expression in long-lived post-mitotic cells (e.g., neurons, hepatocytes)

Ex Vivo: Permanent genomic correction in **hematopoietic stem cells,** which self-renew and provide a lifetime supply of healthy progeny

Immune response challenge: In vivo viral delivery can trigger antibody response, effectively "vaccinating" the patient against the vector
High manufacturing costs ($2-4 million)

Sources: Yi et al. Cell and Gene Therapy: Transforming Treatment Paradigms for Patient-Centric Care. Clin Transl Sci. 2025 Dec;18(12):e70430. doi: 10.1111/cts.70430.
Bhagat et al. Gene Therapy: Towards a New Era of Medicine. AAPS PharmSciTech. 2024 Dec 19;26(1):17. doi: 10.1208/s12249-024-03010-6.
Tang R, & Xu Z. Gene therapy: a double-edged sword with great powers. Mol Cell Biochem. 2020 Nov;474(1-2):73-81. doi: 10.1007/s11010-020-03834-3
Anguela XM, & High KA. Entering the Modern Era of Gene Therapy. Annu Rev Med. 2019 Jan 27;70:273-288. doi: 10.1146/annurev-med-012017-043332.

Gene Therapy FDA Approved For Clinical Use

Category	Product	Delivery Method	In vivo / Ex vivo	FDA Approval Year	Approx. Price Tag (US)	Key Indication
Gene therapy	**Zolgensma** onasemnogene abeparvovec-xioi	AAV9 IV infusion	In vivo	2019	~$2.1–2.5M	SMA (infants)
	Luxturna voretigene neparvovec-rzyl	AAV2 subretinal	In vivo	2017	~$850k per treatment*	Inherited retinal dystrophy
	Elevidys delandistrogene moxeparvovec-rokl	AAVrh74 IV infusion	In vivo	2023	~$3.2M	Duchenne muscular dystrophy
	Hemgenix etranacogene dezaparvovec-drlb	AAV5 IV infusion	In vivo	2022	~$3.5M	Hemophilia B
	Roctavian valoctocogene roxaparvovec-rvox	AAV5 IV infusion	In vivo	2023	~$3M	Hemophilia A
	Zynteglo betibeglogene autotemcel	Lentiviral HSCs	Ex vivo	2022	~$2.8M	Beta thalassemia
CAR T-cell	**KYMRIAH** tisagenlecleucel	Lentiviral CAR T	Ex vivo	2017	~$373k–$475k‡	ALL & LBCL
	YESCARTA axicabtagene ciloleucel	Retroviral CAR T	Ex vivo	2017	~$373k–$538k	LBCL & FL
	TECARTUS brexucabtagene autoleucel	Retroviral CAR T	Ex vivo	2020	~$373k–$490k	MCL & ALL
	ABECMA idecabtagene vicleucel	Lentiviral CAR T	Ex vivo	2021	~$400k–$470k§	Multiple myeloma
	CARVYKTI ciltacabtagene autoleucel	Lentiviral CAR T	Ex vivo	2022	~$400k–$470k§	Multiple myeloma

Sources: FDA, Drugs.com, TechTarget, DrugTimes, Cancer.gov, Brookings

OSHP
OREGON SOCIETY OF HEALTH-SYSTEM PHARMACISTS

Currently FDA approved:

8 in vivo gene therapies

5 ex vivo gene therapies plus 7 CAR T-cell therapies

1 gene editing therapy

World-wide: about 52 gene and CAR T-cell therapies

Gene Therapy Example: ZOLGENSMA

(onasemnogene abeparvovec-xioi)

Most currently used gene therapies fall into four disease areas:

- Neuromuscular diseases (SMA, Duchenne)
- Blood disorders (hemophilia, sickle cell disease)
- Rare metabolic diseases
- Inherited blindness

Spinal muscular atrophy (SMA): Rare muscular disorder

Loss of function mutations in both alleles of the *survival motor neuron 1* gene (*SMN1*)

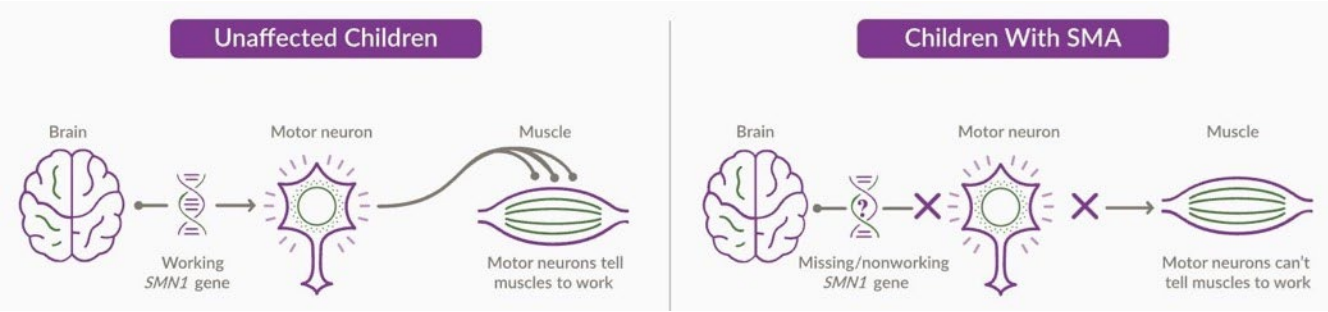
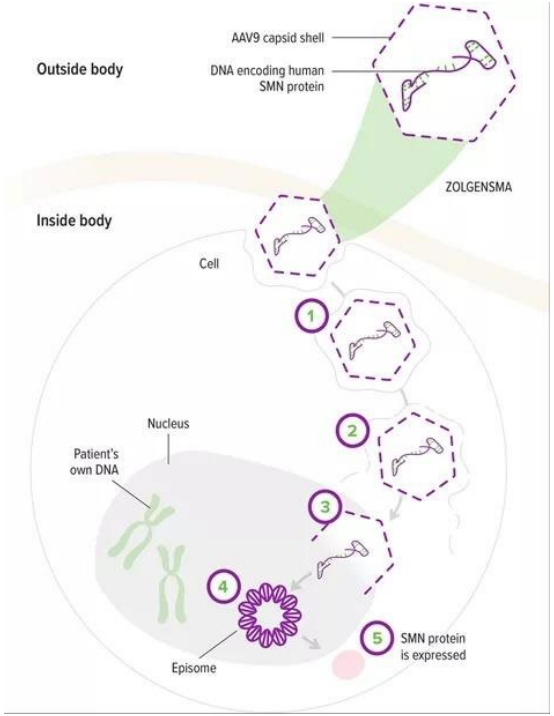


Image source: zolgensma.com/how-zolgensma-works

Gene Therapy Example: ZOLGENSMA

Spinal muscular atrophy (SMA): Rare muscular disorder



Zolgensma (Onasemno**gene** Abe**parvovec**-xioi): **Adenovirus-Associated Virus (AVV9) delivery of *SMN1* gene** to treat children less than two years of age with SMA

Price tag: ~2.5 million

FDA approved 2019

After intravenous infusion, the AAV9 vector passes the blood brain barrier and transduces motor neurons

Viral DNA remains episomal (nucleus) and produces SMN protein

- **Liver Tropism & Toxicity:** Monitoring liver enzymes pre- and post-infusion; liver is a primary site for AAV uptake, **high viral load** can stress the liver and trigger elevated liver enzymes (ALT, AST)
- **Immune Modulation:** Mandatory **prophylactic corticosteroids** begin 24h pre-infusion and continue for ≥30 days to suppress T-cell responses against the viral capsid
- **Systemic Monitoring:** Track **platelet counts and Troponin-I** to screen for Thrombotic Microangiopathy (TMA) and adverse cardiac events.

Sources: www.zolgensma.com

Zolgensma package insert: www.fda.gov/media/126109/download

Gene Therapy Example: Luxturna

(voretigene neparvovec-rzyl)

Leber Congenital Amaurosis (LCA): rare inherited retinal degeneration

RPE65 mutation causes retinoid isomerase deficiency leading to progressive photoreceptor loss in children and severe visual impairment

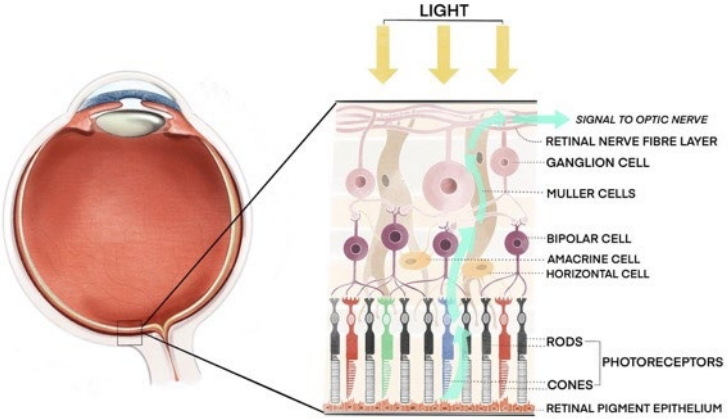
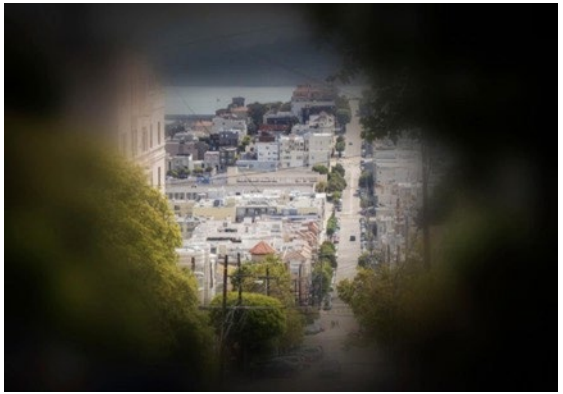
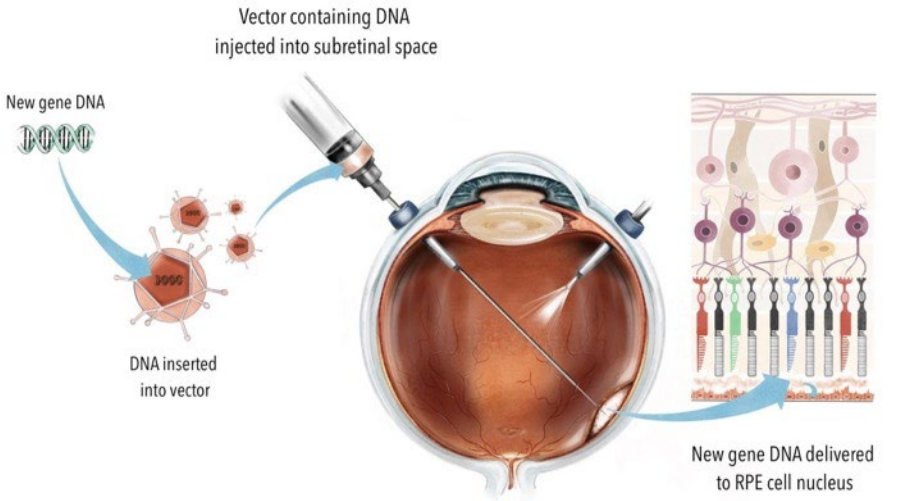


Image source: gene.vision/knowledge-base/rpe65-for-patients/

Typical symptoms: night blindness, tunnel vision



Gene Therapy Example: Luxturna



(RPE: retinal pigment epithelium)

Image source: gene.vision/knowledge-base/rpe65-for-patients/

Localized therapy: 1.5 x 10^{11} vector genomes administered by subretinal injection

Sources: Luxturna.com
Luxturna package insert: www.fda.gov/media/109906/download

Luxturna
(Voretigene neparvovec-rzyl)
Adenovirus-Associated Virus (AVV2) delivery of *RPE65* gene to infants ≥ 12 months old (older children/adults can be treated if retinal cells remain viable)
Price tag: ~$425,000 per eye
FDA approved 2017

Toxicity & Safety Profile

- **Ocular Adverse Events:** Common issues include redness, cataracts, and intraocular pressure
- **Serious Risks:** Retinal tears, macular holes, or severe infection (endophthalmitis)
- **Long-term Monitoring:** Annual monitoring is crucial for injection site risks
- **Systemic Toxicity:** Minimal, as therapy is localized to the eye

Gene Editing Technologies

- Gene or Genome Editing with engineered nucleases is a powerful new approach to:
    - Disrupt or knockout genes
    - Replace genes
    - Correct errors in DNA sequence
    - Silence or increase gene expression
    - Detect DNA sequences
- Genetically engineered sequences are used to target specific regions of genomic DNA
- These sequences are tethered to a nuclease capable of cutting the DNA, usually resulting in a double-strand break

Gene Editing Commonly Used Endonucleases

Zinc Finger Nucleases (ZFNs)

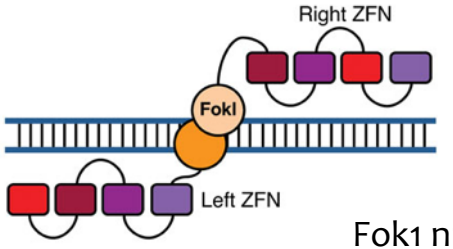
- Zinc finger protein guide Fok1 nuclease to a specific DNA sequence

TALENs or Transcription activator-like effector nucleases

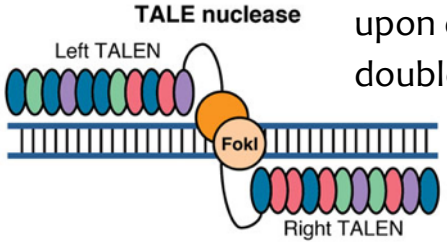
- Transcription activator-like effector guide Fok1 nuclease to a specific DNA sequence

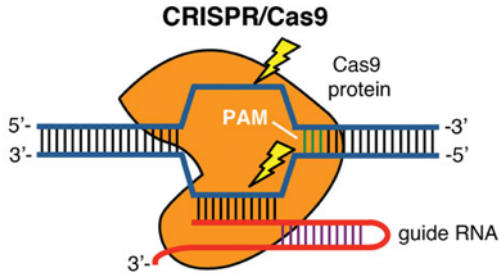
CRISPR/Cas9

- Cas9 nucleases bound by tracRNA that guides complex to specific sites where a double strand break (Cas9) or single strand break (nCas9) is induced



Fok1 nuclease is activated upon dimerization and induces double strand break





CRISPR = Clustered Regularly Interspaced Short Palindromic Repeats

Image source: https://www.genengnews.com/insights/comparing-genome-editing-technologies/

Comparison Of Gene Editing Approaches

	ZFN	TALEN	CRISPR/Cas9
Experiment design and construction	Complex	Complex	Simple
Efficiency of editing	Limited (10-30%)	Average (20-60%)	Good (90+%)
Specificity towards gene target	High	Highest	Moderate
Ability to multiplex	Difficult	Limited	Excellent
Cost of approach (time, $ investment)	Expensive	Expensive	Low

Sources:
Gaj et al. (2013). ZFN, TALEN, and CRISPR/Cas-based methods for genome engineering. Trends in biotechnology, 31(7), 397–405.
Cui et al.. (2021). The comparison of ZFNs, TALENs, and SpCas9 by GUIDE-seq in HPV-targeted gene therapy. Molecular therapy. Nucleic acids, 26, 1466–1478.

CRISPR Gene Editing Therapies in Clinical Trials

NTLA-2001 (nexiguran ziclumeran or nex-z)

- Treatment for Transthyretin (ATTR) Amyloidosis, condition where misfolded proteins cause cardiomyopathy (CM) and peripheral neuropathy (PN).
- Single dose therapy, in vivo delivered via lipid nanoparticles (LNPs) that travels to the liver and use CRISPR-Cas9 to permanently deactivate the *transthyretin* (*TTR*) gene.
- Initial Phase 1 data showed a mean % reduction in serum TTR protein levels of 89% after a single infusion. This effect has proven durable over several years of follow-up.
- Currently in Phase 3 trials (MAGNITUDE)

Sources:
Fontana et al. (2024). CRISPR-Cas9 Gene Editing with Nexiguran Ziclumeran for ATTR Cardiomyopathy. The New England journal of medicine, 391(23), 2231–2241.
Gillmore et al. (2025). Nexiguran Ziclumeran Gene Editing in Hereditary ATTR with Polyneuropathy. The New England journal of medicine, 393(14), 1375–1386.
Fontana et al. (2026). Transthyretin amyloid cardiomyopathy: from cause to novel treatments. European heart journal, 47(1), 54–63

CRISPR Gene Editing Therapies in Clinical Trials

NTLA-2002 (lonvoguran ziclumeran or lonvo-z)

- Treatment for Hereditary Angioedema, a rare genetic disorder that causes tissue swelling due to bradykinin build up and can be life-threatening.
- Single dose therapy, in vivo delivered via lipid nanoparticles (LNPs) that uses CRISPR-Cas9 to knockout the *KLKB1* gene in the liver and reduce plasma kallikrein levels.
- Phase 1/2 results showed significant reduction in plasma kallikrein levels (~90% reduction) and ~80% decreased frequency of monthly swelling attacks compared to placebo, with many patients becoming completely attack-free.
- Currently in Phase 3 trials (HAELO) with results expected in 2026.

Sources:
Cohn et al. (2025). CRISPR-Based Therapy for Hereditary Angioedema. The New England journal of medicine, 392(5), 458–467.
Jalal et al. (2025). CRISPR-Cas9 gene editing for hereditary angioedema: current treatments and emerging therapies. Annals of medicine and surgery (2012), 87(12), 8671–8677.

CRISPR Gene Editing Therapies in Clinical Trials

CTX310

- Treatment for hypercholesterolemia (familial or nonfamilial), moderate-to-severe hypertriglyceridemia, or mixed dyslipidemia.
- Single dose therapy, in vivo delivered via lipid nanoparticles (LNPs) that uses CRISPR-Cas9 to target the *ANGPTL3* gene switching off angiopoietin-like protein 3 expression.
- Recent, preliminary data has shown significantly lowered lipid levels (up to 86% for LDL-C and 84% for triglycerides) with results sustained through 60 days and no significant adverse events.
- Indicates that CRISPR-Cas9 therapies may be used in future to highly prevalent diseases, not just rare genetic disorders

Source:
Laffin et al. (2025). Phase 1 Trial of CRISPR-Cas9 Gene Editing Targeting ANGPTL3. The New England journal of medicine, 393(21), 2119–2130.

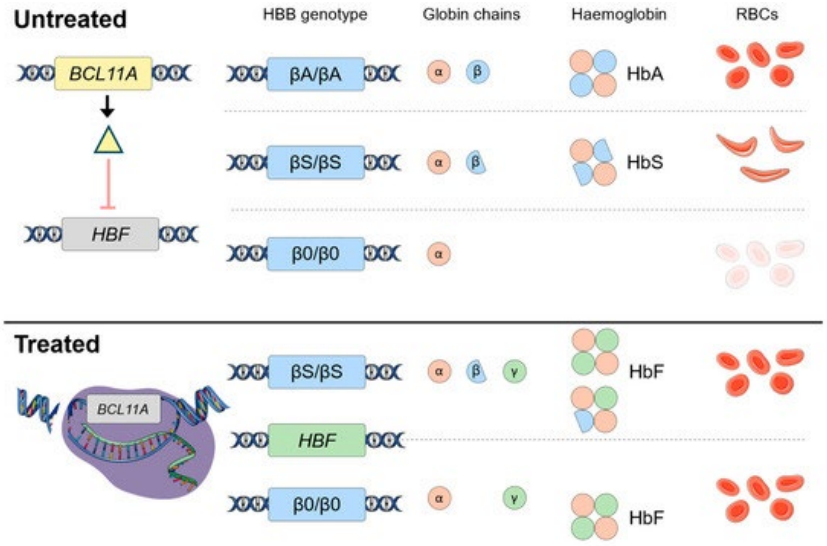
FDA-Approved CRISPR Gene Editing Therapy

CASGEVY® (exagamglogene autotemcel or exa-cel)

- First FDA-approved CRISPR-based therapy
- Used for treatment of Sickle Cell Disease and Beta-Thalassemia
- Single dose treatment with exa-cel (preceded by myeloablation) in 97% of patients with SCD (12-35 yo) eliminated vaso-occlusive crises for a period of 12 months and significantly longer.
- Treatment with exa-cel, preceded by myeloablation, in 91% patients with transfusion-dependent β-thalassemia (12-35 yo) resulted in transfusion independence.
- Phase 3 clinical trials to evaluate exa-cel in pediatric patients (2-11 yo) ongoing and data for 5-11 yo cohort showing similar efficacy and safety data compared to 12-35 yo cohort.

Sources:
Frangoul et al. (2024). Exagamglogene Autotemcel for Severe Sickle Cell Disease. The New England journal of medicine, 390(18), 1649–1662.
Locatelli et al. (2024). Exagamglogene Autotemcel for Transfusion-Dependent β-Thalassemia. The New England journal of medicine, 390(18), 1663–1676
Frangoul et al. (2025). First results of exagamglogene autotemcel in pediatric patients aged 5-11 years with transfusion-dependent β-thalassemia or sickle cell disease with recurrent severe vaso-occlusive crises. Blood, 146 (S1), 379.

Mechanism of Action of CASGEVY®
(exa-cel)



Source: Kerwash et al. (2024). Regulatory Assessment of Casgevy for the Treatment of Transfusion-Dependent β-Thalassemia and Sickle Cell Disease with Recurrent Vaso-Occlusive Crises. Current Issues in Molecular Biology, 46(8), 8209-8225.

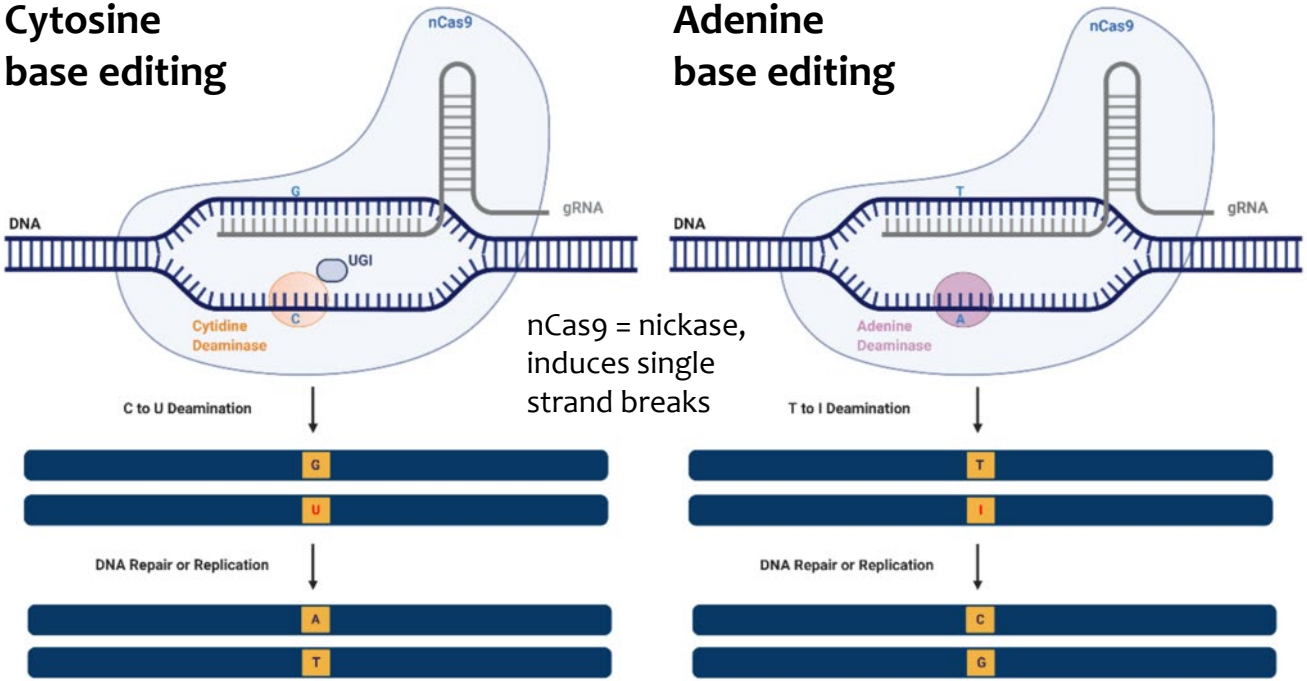
Situation:

- Healthy adults express normal α- and β-globin chains (HbA) and produce healthy erythrocytes
- Minimal fetal hemoglobin (HbF) is present in healthy adults because BCL11A transcription factor suppresses expression
- In SCD or β-Thalassemia, expression α-globin expression is normal, but β-globin is mutated or reduced

Treatment:

- *BCL11A* gene in patient's CD34+ hematopoietic stem cells and hematopoietic progenitor cells is inactivated by CRISPR/Cas9
- Modified CD34+ cells infused back into patient, which produce red blood cells in which suppression of HbF is relieved, enabling replacement of mutated or absent β-globin

Advances in CRISPR-Cas9 Editing Therapeutics: **Base Editing**



nCas9 = nickase, induces single strand breaks

Applications

Correction of disease causing single nucleotide variants

Introduction of point mutations to alter how genes are expressed

Source: Kantor, A., McClements, M. E., & MacLaren, R. E. (2020). CRISPR-Cas9 DNA Base-Editing and Prime-Editing. International Journal of Molecular Sciences, 21(17), 6240.

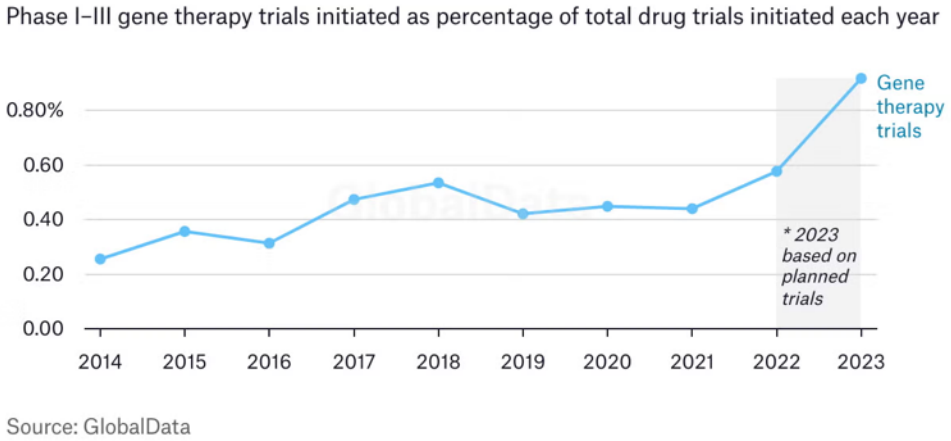
CRISPR Gene Editing Therapies in Clinical Trials

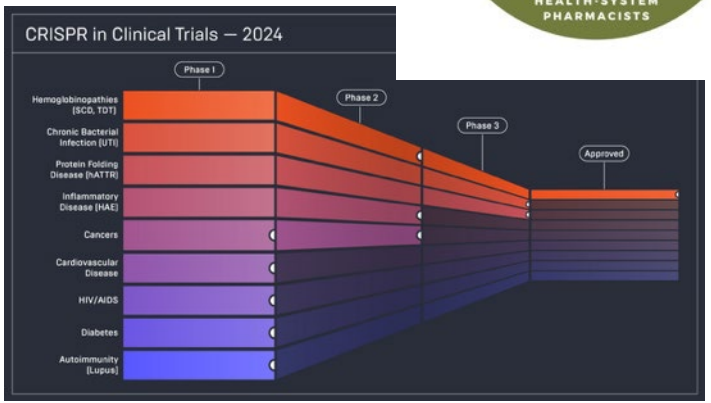
BEAM-302, Example of Adenine Base Editing

- Alpha-1 Antitrypsin Deficiency (AATD) inherited disease causes early onset emphysema and liver disease due to low circulating levels of functional protein
- Most common form arises from AAT-Z mutation, an E342K mutation (GAG to AAG) which causes AAT to misfold and accumulate in liver
- BEAM-302 uses an Adenine Base Editor (ABE) CRISPR-nCas9 delivered via lipid nanoparticle infusion to liver, editing A-to-G and correcting disease causing AAT-Z to AAT-M (wild-type and functional form)
- Phase 1/2 trial, a single 60 mg dose achieved durable AAT-M levels well above the protective threshold and reduced toxic Z-AAT by 84%, suggesting a permanent correction of the genetic cause of AATD

Sources:
Beam Therapeutics Announces Compelling Updated Clinical Data from the Ongoing Phase 1/2 Trial of BEAM-302 in Alpha-1 Antitrypsin Deficiency (AATD) to Support Advancement to Pivotal Development." Investor Relations Press Release, March 25, 2026. Available at: https://investors.beamtx.com
Beam Therapeutics Inc. (2024–2026). A study to evaluate the safety and efficacy of BEAM-302 in adult patients with alpha-1 antitrypsin deficiency (AATD) (ClinicalTrials.gov Identifier NCT06389877). U.S. National Library of Medicine. https://clinicaltrials.gov/study/NCT06389877

Growth in Gene Therapy and Gene Editing Clinical Trials





- A search of clinicaltrials.gov on March 24, 2026 for clinical trials "not yet recruiting", "recruiting", or "active and not recruiting" revealed 1,118 underway for gene therapy and <100 for gene editing.

Image sources:
https://www.clinicaltrialsarena.com/wp-content/uploads/sites/22/2023/01/8X0aC-gene-therapy-trial-initiations-are-on-the-rise2-e1674224165688.png
https://innovativegenomics.org/news/crispr-clinical-trials-2024/

Pharmacist's Role in Next-Gen Medicine

Patient Education: Counsel on therapy action, required follow-up, long-term effects, and reproductive safety

Complex Logistics: Manage preparation, handling, ultra-cold storage (e.g., Luxturna (voretigene neparvovec-rzyl)), and time-sensitive administration (e.g., Zolgensma (onasemnogene abeparvovec-xioi), Casgevy (exa-cel))

Regimen Management: Coordinate pre-treatment (e.g., lymphodepletion, myeloablative conditioning), concomitant therapy (e.g., corticosteroids), and post-treatment monitoring

Safety Monitoring: Oversee adverse effects, long-term risks (e.g., insertional mutagenesis, CRS, ICANS), treatment failure, and registry participation

Patient Selection & Pharmacogenomics: Verify eligibility, interpret genetic data, and integrate genomic results into treatment decisions

Access & Cost: Navigate complex reimbursement for high-cost therapies ($2–4M), including prior authorization, appeals, and specialty pharmacy coordination

Sources: Riedy et al. (2025). Navigating clinical and operational challenges of adeno-associated virus gene therapy: A guide for pharmacists. Journal of the American College of Clinical Pharmacy, 8(6), 469–480.
Shi et al. The role of hospital pharmacists in supporting the appropriate and safe use of CGT/ATMPs: a scoping review of current insights. BMC Health Serv Res. 2025 Jan 9;25(1):52. doi: 10.1186/s12913-024-12026-4.
Halpern L et al. Pharmacists' Critical Role in Gene Therapy: Education, Collaboration, and Patient Care. Pharmacy Times. 2025 April 8.
DiPiro et al. (2025). ASHP and ASHP Foundation Pharmacy Forecast 2025: Strategic planning guidance for pharmacy departments in hospitals and health systems. American Journal of Health-System Pharmacy, 82(2), 17–47.

Breaking Barriers and Unlocking Potential

Barriers to overcome:

- **Delivery challenges:** efficiency, tissue-specific targeting
- **Safety concerns:** insertional mutagenesis, immune reactions, off-target effects
- **Cost & access:** high treatment costs, manufacturing complexity
- **Durability & variability:** inconsistent efficacy across patients
- **Regulatory and ethical hurdles:** long-term follow-up, germline implications

Promises for the Future: A New Era of Medicine

- **Curative Shift:** Moving from chronic symptom management to durable, single-intervention outcomes
- **Next-Gen Delivery:** Advancing toward safer, tissue-specific, non-viral vectors (e.g., LNPs)
- **In Vivo Revolution:** Using Base and Prime Editing to reduce off-target risks
- **Expanded Reach:** Moving beyond ultra-rare monogenic diseases to high-prevalence areas (cardiovascular, autoimmunity, oncology)
- **Pharmacy Stewardship:** Establishing the pharmacist as the Genomic Gatekeeper for patient selection, PGx-driven conditioning, and surveillance

Post-Test Questions

1. **Which of the following best distinguishes a "gene editing" therapeutic approach from traditional gene therapy? Gene editing:**
   A. requires periodic re-administration to maintain the therapeutic effect.
   B. uses a nuclease to permanently modify an endogenous DNA sequence.
   C. relies exclusively on viral vectors to deliver content to the nucleus.
   D. introduces a functional gene copy without altering the host genome.

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Post-Test Questions

2. **Which of the following represents a significant "promise" of gene editing in the context of pharmaceutical treatment strategies?**

   A. Transitioning from chronic symptom management to a "one-and-done" curative approach
   B. Immediate reduction in the Total Cost of Care (TCC) for the first year of treatment
   C. May be delivered via community-based speciality pharmacies with standard cold-chain storage
   D. Minimizing the requirement for long-term patient monitoring

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3. **As of 2026, which of the following is an FDA-approved gene editing therapy utilized for the treatment of Sickle Cell Disease and Beta Thalassemia?**

   A. Voretigene neparvovec (Luxturna)
   B. Onasemnogene abeparvovec (Zolgensma)
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Thank you!
Any questions?