CRISPR and the Future of Genetic Medicine

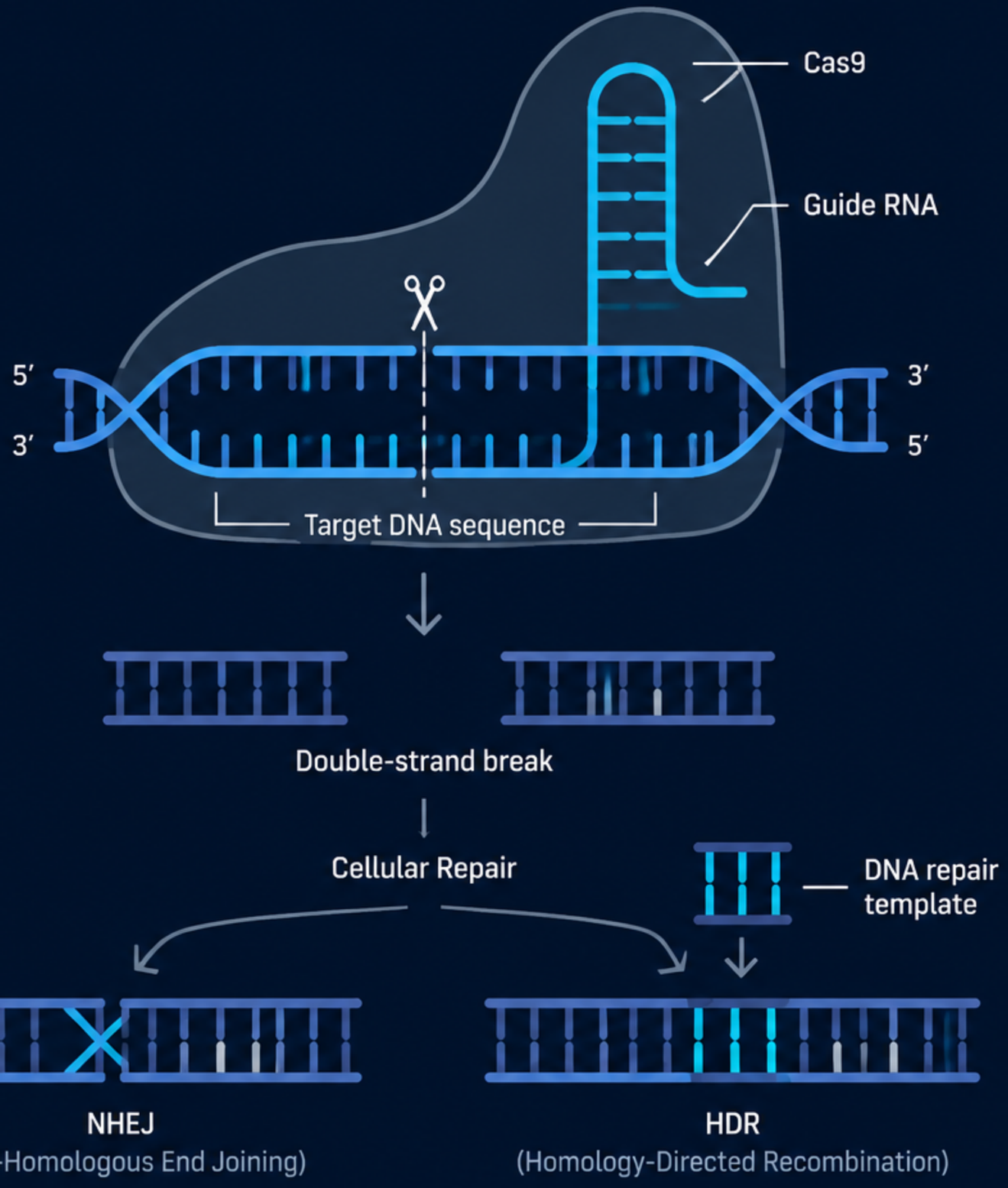
From Molecular Scalpel to Clinical Reality

- A comprehensive overview of CRISPR technology
- Current clinical trials and approved therapies
- Ethical considerations and the path forward

The Molecular Scalpel

How CRISPR-Cas9 Edits the Genome

- Guide RNA locates the specific target DNA sequence.
- Cas9 enzyme acts as molecular scissors to cut the DNA.
- The cell's natural repair mechanisms are harnessed to add, remove, or alter genetic material.



The Clinical Landscape

Unprecedented Growth in Gene-Editing Trials

~250

Monitored Clinical Trials

>150

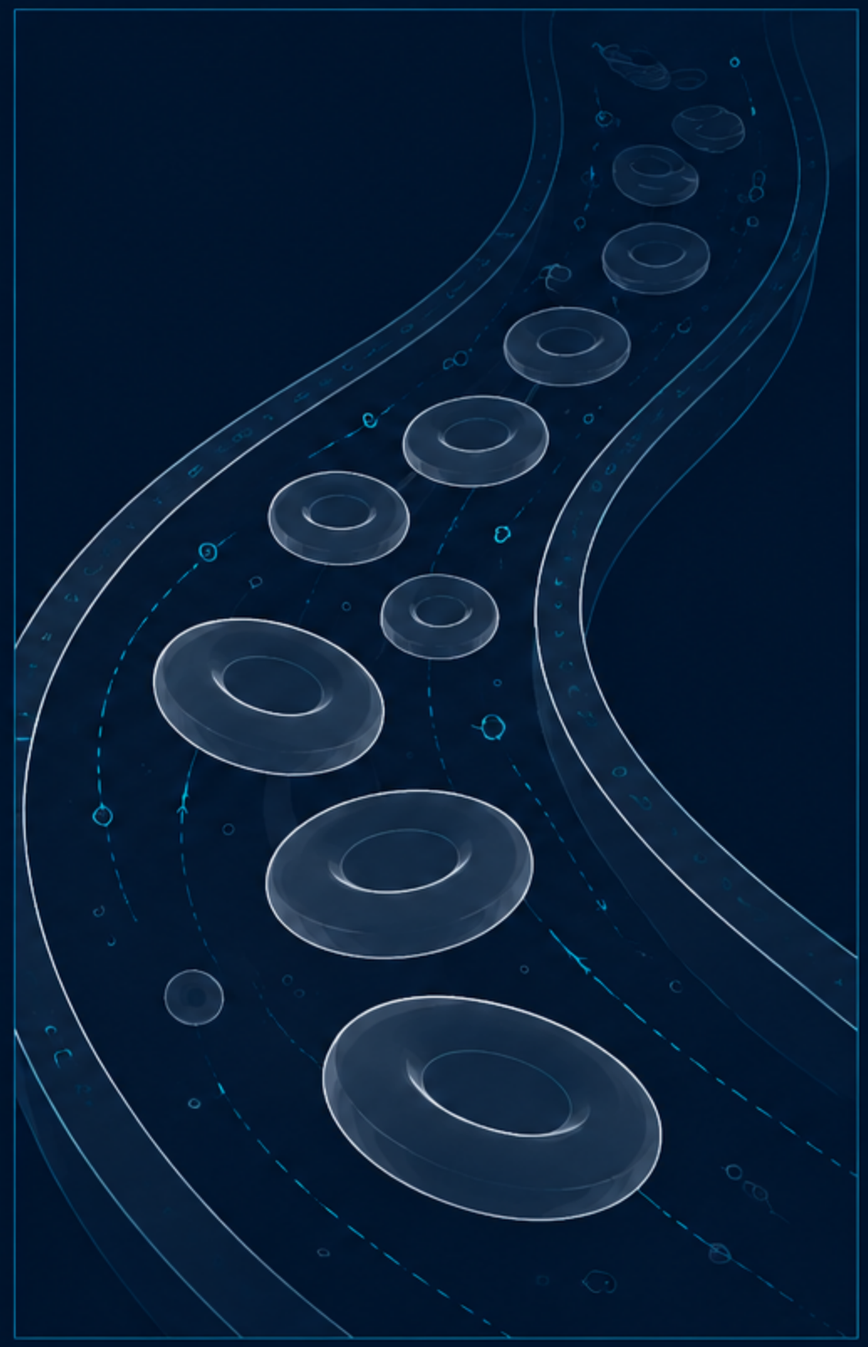
Active Therapeutic Candidates

- As of early 2025, CRISPR Medicine News monitors ~250 clinical trials.
- Over 150 of these involve active gene-editing therapeutic candidates.
- Rapid expansion from early-stage research to applied human therapeutics.

First Approved Therapies

Milestones in Hemoglobinopathies

- The historic approval of Casgevy (exa-cel) marked the first CRISPR-based therapy.
- Targets inherited blood disorders: Sickle Cell Disease and Transfusion-Dependent Beta Thalassemia.
- Developed by Vertex Pharmaceuticals and CRISPR Therapeutics.



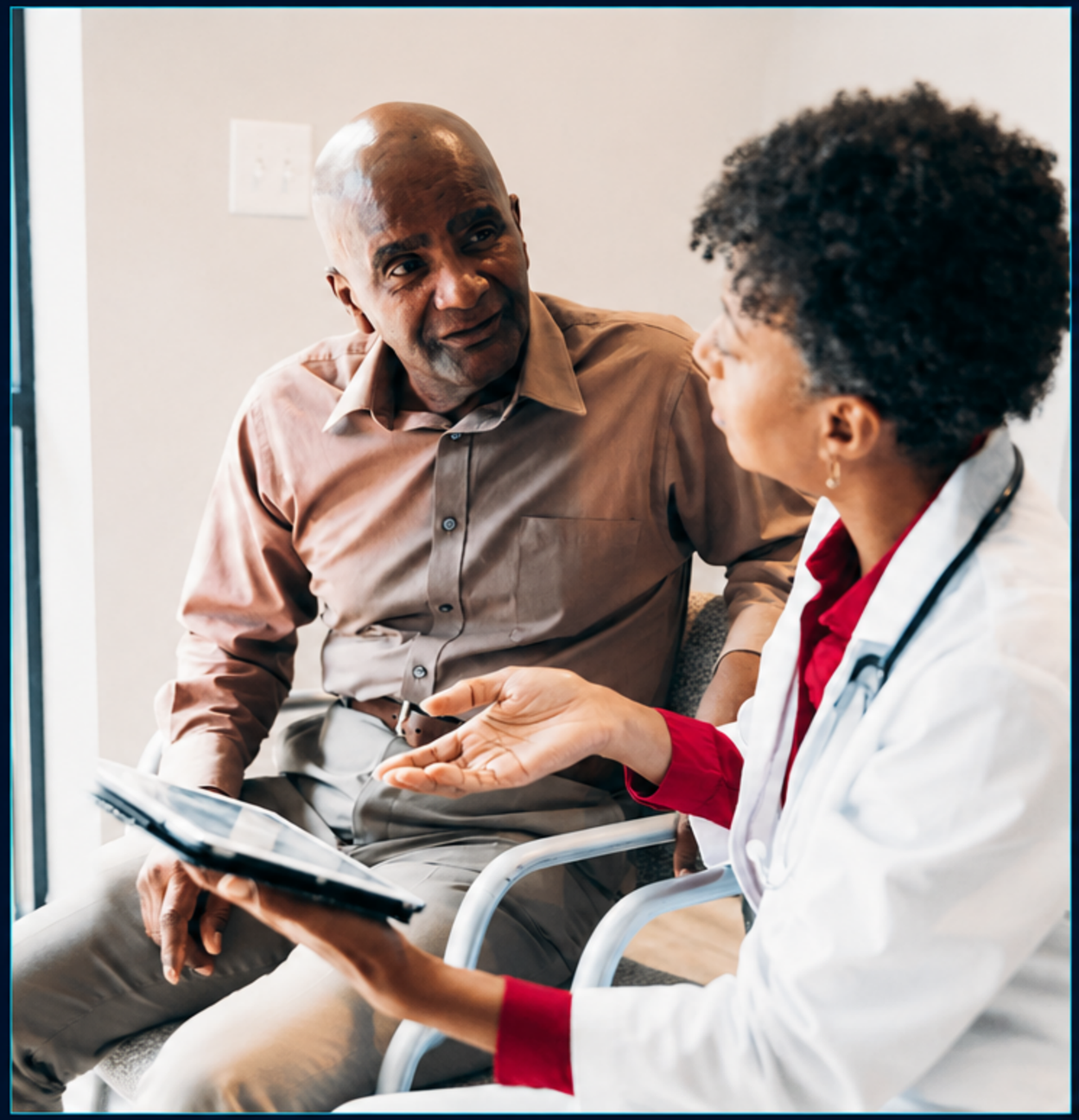
Transforming Sickle Cell Disease

From Lifelong Management to Curative Potential

Modifies a patient's own hematopoietic stem cells to produce high levels of fetal hemoglobin.

Eliminates severe vaso-occlusive crises for many patients.

Requires complex, intensive pre-treatment and specialized medical infrastructure.



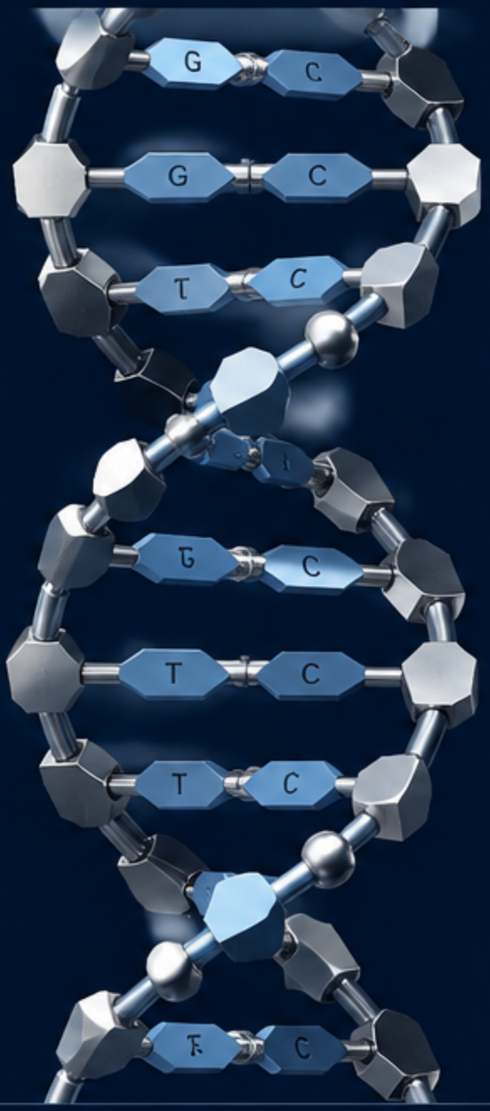
Somatic vs. Germline Editing

The Crucial Biological Distinction

Somatic Editing

Changes target non-reproductive cells.

Effects are confined to the patient and are not passed to offspring.



Germline Editing

Modifies embryos, sperm, or eggs.

Changes become heritable and affect all future generations.

Ethical Considerations

Navigating the Implications of Genome Editing

Concerns regarding unequal access to expensive, life-altering therapies.

Fears of genetic enhancement and exacerbating global inequalities.

Broad consensus against human germline editing for reproductive purposes due to unknown generational risks.

Expanding Horizons

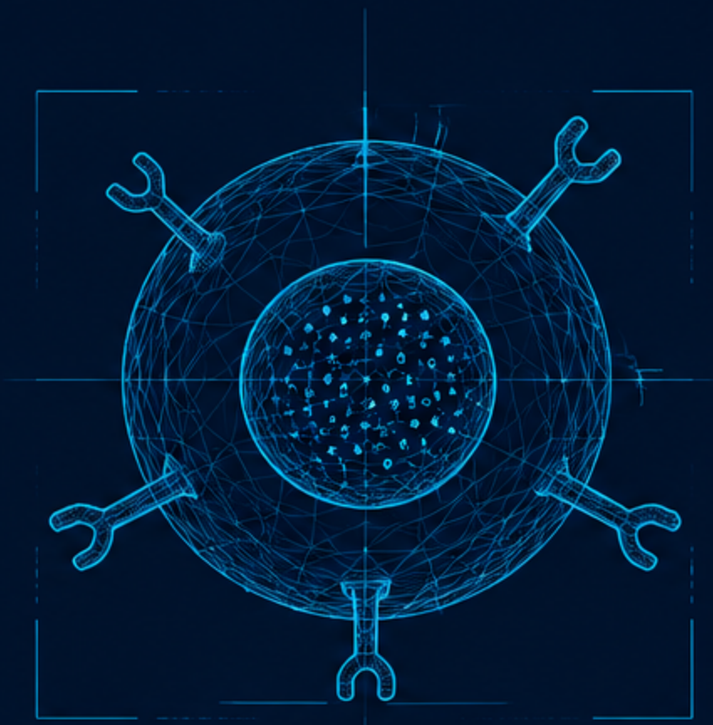
Targeting Cancer and HIV

Oncology

Engineering CAR-T cells using CRISPR to create more potent, 'off-the-shelf' cancer immunotherapies.

Infectious Disease

Ongoing research explores CRISPR's ability to excise HIV proviral DNA directly from infected human cells.



Future Frontiers

Cardiovascular Disease and Type 1 Diabetes

- In-vivo editing approaches are being developed to permanently lower cholesterol and treat cardiovascular disease.

- Research is advancing toward using CRISPR to create immune-evasive, insulin-producing cells for Type 1 Diabetes.

- Moving from ex-vivo (outside the body) to in-vivo (inside the body) delivery systems.

The Future is Now

Balancing Innovation with Responsibility

- CRISPR has rapidly evolved from a biological curiosity to a clinical reality.
- Continued investment in both delivery mechanisms and ethical frameworks is required.
- The era of programmable genetic medicine has arrived.