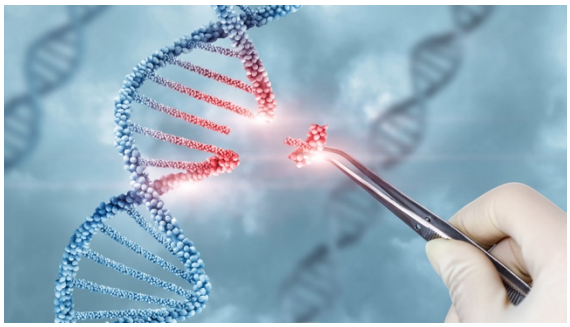
Gene Therapy Breakthroughs Offer New Hope — but Raise Hard Questions About Cost, Access and Fairness



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Gene therapy has rapidly moved from an experimental concept to a realistic medical option for several rare and previously untreatable diseases. By altering or replacing faulty genes, these treatments aim not merely to relieve symptoms but to intervene at the root of the disorder. Yet as breakthroughs accelerate, so too do urgent questions about who will be able to access them, how much health systems can afford, and what the ethical landscape will look like if the most transformative treatments remain available only to a privileged few.

Recent approvals for sickle cell disease in the United States illustrate both the promise and the barriers of this new era. Two one-time gene therapies—Casgevy and Lyfgenia—can prevent the painful crises and life-limiting complications that define the condition. The procedures require extracting a patient's own stem cells, genetically modifying them, and reinfusing them after intensive chemotherapy. For people who have spent decades facing limited treatment options, the possibility of a durable cure is extraordinary. Yet each therapy carries a price tag of more than two million dollars, and both depend on access to highly specialised transplant centres.

In countries where sickle cell disease is most common—particularly in Nigeria, the Democratic Republic of Congo, and other parts of sub-Saharan Africa and South Asia—such clinical infrastructure is scarce. Nigeria alone accounts for nearly 50% of the world's sickle cell births each year, yet it has fewer than 10 fully equipped stem-cell transplant centres for a population of over 220 million people. The World Health Organization has noted that "three-quarters of children with sickle cell disease in sub-Saharan Africa die before the age of five due to lack of diagnosis and treatment." As Dr. Kolawole Oyedeji, a Nigerian haematologist, explains, "We are talking about countries where even basic antibiotics are hard to access. The idea of a multi-million-dollar gene therapy here is unimaginable for most patients."
Similarly, Dr. Julie Makani from Tanzania, one of Africa's leading sickle-cell researchers, has stated, "The science is moving faster than the health systems that need to deliver it. Without investment in laboratories, training and long-term care, gene therapy will remain out of reach for the majority of African patients."

Even within wealthy nations, experts warn these breakthroughs may widen existing inequalities, as the communities most affected by the disease—often minority or economically disadvantaged groups—already struggle to access specialised care.

A similarly complex story is emerging in Europe, where early clinical trial results for a novel gene therapy for Huntington's disease have generated cautious optimism. A small, first-in-human study has reported a striking slowing of progression after a single neurosurgical procedure delivering genetic material deep into the brain. Researchers have described the findings as among the most promising in the history of Huntington's disease research.

Yet the obstacles are considerable. The therapy requires hours-long brain surgery, advanced imaging facilities, and highly trained neurosurgeons. Only a handful of hospitals in the UK and across Europe are currently capable of delivering such a procedure. Although no official price has been announced, analysts expect the therapy to cost several million pounds—consistent with other rare-disease gene therapies. Even with regulatory approval, access would almost certainly be limited. National health systems such as the NHS will face difficult decisions about which patients qualify, how limited treatment slots are allocated and how to balance investment in rare conditions against broader public health needs. The scientific achievement is remarkable, but innovation alone does not guarantee equitable availability.

Cost lies at the heart of these debates. A recent review found that traditional value-based pricing thresholds—such as $100,000–$150,000 per quality-adjusted life year (QALY)—are incompatible with current gene-therapy prices unless the lifetime benefit is assumed to be far shorter than promised. Another analysis warned that "the current cost of licensed gene therapies is a challenge for high-income countries … and will make widespread availability in low- to middle-income countries all but impossible."

Ethicists have been explicit about the stakes. Philosopher Françoise Baylis argues that “one of the biggest and most realistic risks is that the people who could benefit most won’t be able to access or afford them.” Industry observers share this concern. “These are the most expensive drugs in the world… the whole health system, the pharmaceutical companies are just trying to figure out how to make these drugs accessible,” says Kevin Doxzen of the World Economic Forum.

Examples from outside Europe underscore the point. In the US, single-treatment gene therapies are now priced at $2.8 million and up. In Europe, a recent gene therapy for metachromatic leukodystrophy was launched at $4.25 million in the US and €2.4 million in Germany. Meanwhile, the countries where many rare genetic disorders are most prevalent are effectively excluded from access. As the Associated Press reported, “promising new gene therapies for sickle cell disease are out of reach in countries where they’re needed most.”

These realities demand serious ethical scrutiny. Inequality of access is growing: if cures exist but only wealthy hospitals in wealthy countries can deliver them, the principle of healthcare equity collapses. Financial sustainability is another concern, as national health systems must ask how many millions they can spend on treatments that serve very small patient groups when resources are finite. Prioritisation becomes ethically fraught: when only limited doses or surgical slots are available, who should receive them first? Long-term safety remains uncertain, since these therapies are new, irreversible and lack decades-long data. And at a global level, deep questions of justice arise: why are groundbreaking therapies developed and trialled almost exclusively in high-income countries, even for diseases disproportionately affecting lower-income regions? As one Ugandan researcher put it, “67% of the world’s HIV population is in sub-Saharan Africa … about 40 trials of gene therapy have been conducted globally, and none has happened in a region with the highest prevalence.”

For clinicians, policymakers and ethicists, the implications are enormous. On one hand, gene therapy may rewrite the future of once-fatal diseases like Huntington’s and sickle cell disease. On the other, if these therapies serve only a narrow segment of society, the question is not merely “can we do it?” but “who will receive it?” and “at what cost?” Gene therapy may well define the next era of medicine—but whether it does so equitably remains an open and urgent question.

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Expert commentary from Dr. Kolawole Oyedeji (Nigeria) and Dr. Julie Makani (Tanzania).