Gene Therapy as Preventive Care: Rethinking Payment for One-Shot Cures

Arya S. Rao
Harvard Medical School Class of 2030

Gene therapy has revolutionized modern medicine, enabling transformative treatments for previously incurable diseases. As of 2023, 48 cellular and gene therapies are on the market, including Casgevy, the first FDA-approved CRISPR-Cas9 therapy. Hundreds more are currently in clinical development (1). Physicians will soon be able to wield these molecular scissors towards bespoke therapies for individual patients, promising personalized, one-shot cures for genetic diseases ranging from classic Mendelian disorders to complicated, heterogeneous cancers. Yet, current therapies average millions of dollars per dose, making treatment cost-prohibitive for patients (2).

As gene therapy begins its explosive rise, existing payment and delivery models are struggling to adapt. Medicaid programs are generally required to cover FDA-approved drugs, including gene therapies; however, given their high cost, they are typically subject to stringent prior authorization. Commercial insurance providers are not universally required to cover gene therapies, and current policies tend to restrict the number of patients who may receive gene therapy in a given year (3). Uninsured patients have little hope of meeting the high cost barrier.

Given the potentially curative benefits associated with a just single dose of gene therapy, cost

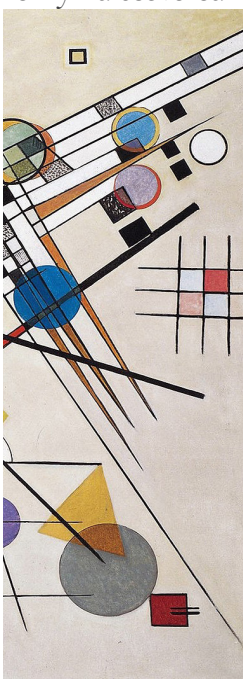
structuring for gene therapies must be adapted to maximally benefit patient wellbeing. The high upfront costs associated with gene therapies disincentivizes their use over traditional, long-term pharmacologic therapies which may offer temporary relief but do not address the root cause of disease. The healthcare system faces a serious challenge to evolve in response.

One novel approach to reimbursement is value-based care (VBC), in which payments are aligned with patient outcomes (4). Whereas the current fee-for-service model rewards a higher volume of services provided, VBC models use outcome

metrics like curative potential, quality of life, and more to determine cost. VBC advocates suggest that pricing gene therapies in this way honors their lifesaving capacity: a one-time cure is worth the million-dollar price tag, weighed against the current standard of care (5).

While VBC models prioritize patient outcomes in theory, they still create high cost barriers for gene therapies, preventing patients from accessing these key cures. **Responsible pricing for gene therapies necessitates a paradigm shift in which preventive care is emphasized in reimbursement models.**

In the United States, providers are paid based on their treatment of a patient's pre-existing or newly discovered condition. Reimbursement correlates with the number of services and/or outcome of services provided. This model has led to reduced uptake of preventive care services— the current system is equipped to handle disease when it happens, not before it happens. Under this model, a gene therapy which can dramatically reduce the burden of a disease on a patient throughout their lifetime is priced according to that burden, requiring cost determinations of the value of a human life via quality-adjusted life-years. Instead, gene therapies can and should be viewed as preventive therapies, which prevent the burden of a disease rather than simply displace it. Accordingly, our healthcare system must shift to account for these new preventive therapies.



This proposition aligns with a broader imperative to prioritize early intervention in our medical system. Preventive care has long been recognized for its potential to significantly reduce long-term healthcare costs by addressing conditions before they escalate, allowing both for reduced lifetime medical expenses and improved outcomes.

Gene therapies can and should be viewed as preventive therapies.

Gene therapies embody this principle, offering the chance to correct genetic anomalies before they manifest as complex medical conditions requiring extensive, continued care.

Already, our system includes provisions for preventive measures which, if applied to gene therapies, could significantly reduce costs. Commercial insurance companies may consider adjusting premiums for gene therapy patients, or might offer incentives for the use of gene therapies akin to wellness incentives for healthy behaviors. On a federal level, the Center for Medicare & Medicaid Services (CMS) can associate cost of gene therapies with population-based risk reduction as opposed to individual patient benefit, grouping gene therapies for rare diseases to reflect the overall population's benefit from these novel cures. CMS has successfully applied similar population health strategies for cardiovascular disease and diabetes prevention (6). Lastly, the federal government could incorporate incentives for pharmaceutical companies to develop preventive therapies, thereby creating a financial incentive for a philosophical shift towards preventive care.

Incorporating gene therapies into preventive care reimbursement frameworks is a necessary step towards fully realizing the patient care benefits of recent groundbreaking progress in genetic engineering. As gene therapy becomes more commonplace and curative solutions for many conditions emerge, it is crucial that payment models evolve in parallel to facilitate the equitable distribution of care. **Framing gene therapies in the broader context of the preventive care movement will improve health equity and make gene therapy a realistic option for patients in need.**

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Correspondence: arya_rao@hms.harvard.edu

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