

CELL AND GENE THERAPY

Cell and gene therapies represent some of the most innovative advances in modern medicine. These highly individualized therapies can treat disease at its source or even cure serious diseases—including rare genetic conditions and aggressive cancers.

Q: What are cell and gene therapies?

Although often discussed together, cell therapy and gene therapy are two distinct approaches:

- **Gene therapy** works by repairing or replacing faulty genes so the body can better fight or cure disease.¹
- **Cell therapy** involves collecting, modifying or reprogramming a patient's or donor's cells to fight disease, then returning them to the body.²

Q: How are they used today?

Cell and gene therapies have moved from concept to clinical reality. For example:

- **Cancer:** CAR T-cell therapies are approved for several leukemias, lymphomas and multiple myeloma, offering remission for patients.³
- **Rare disease:** Gene therapies have restored vision for patients with Leber congenital amaurosis, reduced symptoms for spinal muscular atrophy and cured some sickle cell disease patients.
- **Inherited conditions:** Treatments are being developed for hemophilia, cystic fibrosis and more.

Clinicians report growing optimism as more cell and gene therapies win FDA approval and as long-term data show enduring benefits for patients.



Q: What are the barriers to access?

Access to cell and gene therapies is limited by a mix of financial, logistical and geographic barriers. These treatments can cost millions of dollars per patient, which makes traditional coverage and reimbursement systems difficult to apply. Public and private payers are exploring ways to manage these costs. That can mean paying upfront or in installments.⁴ Sometimes it means tying payments to the therapy's effectiveness through outcome- or value-based agreements.

Beyond cost, the therapies themselves are complex to deliver. The treatment process is highly specialized and time intensive. Access is also influenced by geography, since only select medical centers are equipped to administer these therapies and provide the intensive monitoring they require. For patients in rural or underserved areas, geographic limitations can be just as significant a barrier as financial hurdles.

Barriers to Access: Cell & Gene Therapies



Costs increase up to millions per patient



Traditional insurance models don't fit



Treatment is highly complex and specialized



Requires significant time and expert coordination



Only select clinics can deliver these therapies



Limited access for rural or underserved communities

Q: How can access be improved?

Improving access to cell and gene therapies will require coordinated policy and system-level solutions.

A key priority is developing guidelines to assess the value of these therapies that fully account for the long-term clinical benefits, reduced health system costs and improved quality of life these therapies can provide. Traditional methods of measuring value often overlook these therapies' unique, curative potential. Decisionmakers can improve access through:

- **Allowing for innovative payment models**, such as installment plans and outcomes-based agreements. These models can help balance high upfront costs with long-term benefits and financial sustainability.
- **Expanding the number of treatment centers** through training, accreditation and infrastructure support. This is also essential to address geographic disparities, so patients don't have to travel long distances to receive care.
- **Refining early diagnosis and referral pathways** to help identify eligible patients sooner, increasing the likelihood that they benefit from these transformative treatments.

Q: What role does Medicaid play?

Medicaid covers many patients eligible for gene therapies, particularly those from underserved populations. To expand access, the Centers for Medicare & Medicaid Services, or CMS, launched the Cell and Gene Therapy Access Model, beginning with treatments for sickle cell anemia.⁵ Under this program, CMS works with drug manufacturers and states to set up rebates based on how well the treatments work. This has the potential to get Medicaid beneficiaries life-changing therapies. As more therapies gain approval, Medicaid's role in shaping access and payment will remain essential.

Methods of measuring value should reflect these therapies' **unique, curative potential.**



CONCLUSION

Cell and gene therapies are transforming care for certain diseases and conditions. But innovation alone is not enough. Patients—all patients—need fair and timely access. By aligning innovation with access, policymakers and payers can ensure that breakthroughs in science become breakthroughs in the lives of patients.

SOURCES

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