

How the U.S. Health Care System Can Better Accommodate Cell and Gene Therapies

Biopharmaceutical innovation is ushering in the next generation of advanced medicines. Cell and gene therapies fight diseases at their source—the cellular or genetic level—often through one-time administration, resulting in long-term or potentially curative benefits. As a result, these transformational therapies are widely regarded as revolutionizing health care. For example, we can now cure inherited childhood blindness and help children with spinal muscular atrophy live past the age of two.

Cell and gene therapies work by using an individual's cells and genes for therapeutic use. In gene therapy, a patient's disease-causing genes are replaced, repaired or inactivated to potentially treat, cure or even prevent disease. In cell therapy, cells are cultivated or modified outside the body before being injected into the patient to treat a condition in which the patient's cells are damaged or diseased. Some therapies are considered both cell and gene therapies. For example, CAR T-cell therapies are immune cells that are taken from the patient and genetically modified before being (re)introduced into the patient to fight cancer. While cell and gene therapies use a range of unique and innovative approaches to target a wide range of diseases, collectively they are regarded as among some of the most significant advances in health care, with the potential to dramatically change the trajectory of many devastating, life-threatening conditions for patients.

I Cell and Gene Therapies Offer Tremendous Promise

America's biopharmaceutical companies have invested nearly \$1 trillion in the research and development of new treatments over the past two decades, setting the stage for the next frontier of cutting-edge new treatment approaches. Since the first U.S. Food and Drug Administration (FDA) approved cell-based gene therapy in 2017, these new medical technologies have begun to transform treatment for patients with many devastating illnesses and conditions. There are currently six FDA-approved CAR-T cell therapies and four approved gene therapies available in the U.S. There are also more than 480 cell and gene therapies currently in development to treat a range of diseases and conditions, including cancers, genetic disorders, neurological disorders, cardiovascular disease and others.ⁱ

Importantly, due to their long term and potentially curative effects, cell and gene therapies also hold promise in avoiding the tremendous health care costs associated with the previous standard of care for many burdensome illnesses. Consider hemophilia A, which is associated with a high level of treatment burden, particularly for severe patients, who require administration of blood factor replacement therapy 2-3 times per week in order to prevent dangerous bleeding events over the course of a lifetime. Due to this significant treatment burden, the average health care costs for severe hemophilia patients can range as high as \$760,000 annually, with the most of these costs attributed to blood factor replacement therapy. A range of gene therapies currently in development have demonstrated in clinical trials an almost complete reduction in blood factor replacement therapy utilization in the years following just one administration.ⁱⁱ Likewise, the cost savings potential to the health care system of completely transforming the treatment paradigm of these illnesses, with continued savings offered potentially over the course of a lifetime, is remarkable.

As we prepare for the continued introduction of more cell and gene therapies in coming years, it is also important to recognize that the costs of these treatments are expected to remain affordable and manageable to the health care system overall. One study projected, under a variety of assumptions, that cell and gene therapies will remain a small share of future U.S. health care spending over the next 5-8 years, totaling less than .5% of future health care spending.ⁱⁱⁱ While these costs are manageable, as these therapies have ushered in a new treatment paradigm they bring challenges that are new to stakeholders and our health care system.

I Challenges and Opportunities

We can realize the promise of these therapies and make them accessible and affordable to patients. However, achieving this goal will require a collective effort to advance innovative approaches and move toward a value-driven health care system.

As patients may experience long-lasting or even curative effects from just one administration of many cell and gene therapies, much of this value may only be fully realized over the lifetime of the patient. As such, the current reimbursement system must adapt and evolve to account for the value and potential long-term benefits these groundbreaking treatments deliver to patients.

To address potential access barriers caused by large up-front payments, payers and manufacturers are exploring new ways to finance these therapies. Innovative contracts—also known as value-based contracts or alternative financing arrangements—may help reduce plan risk by tying payment to value as determined by the contracting entities and can help payers manage budget impacts while increasing the focus on patient outcomes and access. Over 100 innovative contracts have been publicly announced that cover nearly 70 brand name medicines from almost 40 biopharmaceutical manufacturers, spanning a range of conditions including multiple sclerosis, diabetes and cancer.^{iv} Some arrangements, like outcomes-based contracts can link payment to patient outcomes while others, like pay-over-time and subscription models, may help payers and patients by shifting risk onto biopharmaceutical manufacturers. Research shows that these types of contracts can potentially increase patient access to critical medications, improve treatment management and care coordination, reduce health system costs and patient out-of-pocket costs and improve patient access and health outcomes.^{v, vi}

Payers and insurers are also finding ways to manage budget impacts through reinsurance and stop-loss policies. As the market evolves, insurers, pharmacy benefit managers and specialty pharmacies are offering products and services that provide diverse approaches to addressing stakeholder needs including contract negotiation and data management, provider contract negotiation and financial and pharmaceutical company warranty services.^{vii} State Medicaid programs are also seeking novel solutions for affordability concerns, including modified subscriptions, population-based models and outcomes-based models.

Despite these advancements, outdated government policies can create uncertainty around innovative payment approaches, which may deter biopharmaceutical companies, insurers and other risk-based providers from adopting or further expanding their move toward innovative contracts. This can potentially limit the number, type and scope of these arrangements. For example, uncertainty in the scope and application of federal anti-kickback rules can inadvertently discourage beneficial low-risk health care arrangements through the threat of civil, criminal and administrative sanctions. Updating these federal rules—in a way that maintains strong protections for patients—will help encourage the use of innovative payment arrangements and help ensure patients can access the therapies they need.

Complex price reporting rules also present a significant barrier. While the Centers for Medicare & Medicaid Services' (CMS) Medicaid value-based purchasing final rule contains new reporting flexibilities, including the reporting of multiple best prices under value-based purchasing agreements, further efforts and clarity may be required to reduce barriers to innovative contracts across a broader range of therapeutic areas. CMS should continue efforts to modernize price reporting for innovative contracts in response to requests from manufacturers.

As noted by the FDA, some of these therapies “are almost certainly going to change the contours of medical practice, and the destiny of patients with some debilitating diseases.” It is critical that we work together to develop a coverage and payment system that ensures timely patient access, manages short-term affordability challenges while continuing to foster the development of these new treatments.

I PhRMA analysis of Adis R&D Insight database.

II PhRMA. [Potential Gene Therapies Hold Promise for Transforming the Trajectory of Many Blood Disorders and in Reducing Significant Treatment Burden and Costs](#). 2022.

III Young CM et al. *Drug Discovery Today*, 27(1): 2022.; CMS National Health Expenditures (NHE) Projections, 2022.; Altarum Institute. “Projections of the Non-Retail Prescription Drug Share of National Health Expenditures.” September 2020.

iv PhRMA, [Innovative Contracts 2009 - 2021](#). April 2022.

v Verpora. [Understanding the Patient and Caregiver Benefit of Value-Based Agreements in the U.S.](#) October 2020.

vi National Pharmaceutical Council. [Value-Based Contracts](#).

vii MIT NEWDIGS, [Emerging market solutions for financing and reimbursement of durable cell and gene therapies](#), June 2021.

