

## Overview

As the House and Senate examine the accessibility of medicines, one concept continues to be largely absent from the conversation: gene therapy. Researchers and drug manufacturers are spending billions of dollars to develop highly-specialized therapies to cure diseases or extend the life of patients who are out of options. Yet, as the Food and Drug Administration (FDA) has begun approving more of these treatments, health industry leaders and experts are debating how to handle the influx of those coming to market.

In this Basic, we discuss what gene therapy is and how the healthcare system is preparing to accommodate these new treatments.

## What is gene therapy?

Gene therapy addresses the underlying, genetic cause of a disease to treat or cure a patient. According to the FDA, these medicines typically work by replacing a disease-causing gene with a healthy copy of the gene, inactivating a disease-causing gene that is not functioning properly, or introducing a new or modified gene into the body to help treat a disease. The Center for Biologics Evaluation and Research (CBER) at the FDA is responsible for regulating these products. The first gene therapy in the United States gained FDA approval in 2017 and about 15 other gene or cell therapy products have earned approval since.

Gene therapy can be used to treat inherited disorders, some types of cancer, and certain viral infections and is currently only being tested for diseases that have no other cure or effective treatment. Researchers use “vectors” to deliver the gene, which often include viruses that cannot cause disease when used for a patient, but are instead genetically modified to carry the gene into a cell.

Given this complexity, these therapies are utilized in a medical setting where the vector can be injected intravenously or a sample of the patient’s cells can be removed to expose them to the vector in a laboratory and reintroduced back into a patient. Like most new treatments, there are risks involved; however, for many patients this is their last hope. The FDA closely monitors the clinical trials of these therapies to make determinations about safety and efficacy for patients moving forward. More information on the FDA’s clinical trial process can be found [HERE](#).

## How is it paid for?

As much hope as these treatments hold for patients, they also spur a lot of anxiety for those that worry they will be cost-prohibitive. The price tag for these often one-time treatments can cost hundreds of thousands to millions of dollars. Current treatment protocols for some of these diseases can cost as much or more over a lifetime, but how does the healthcare system handle it when the treatment is administered just once, or only a handful of times?

Americans usually think of prescription drugs in terms of the once-a-day pill or injection they use and pay for up front at the pharmacy. These prescriptions are usually paid for according to a volume-based payment system, or one where the

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In order to meet our challenges we need to put aside the partisan bickering that has gridlocked Washington and come together to find common sense solutions.

For more information, please visit [www.center-forward.org](http://www.center-forward.org)

## Key Definitions

- **Gene therapy:** technique that modifies a person’s gene or alters the biological properties of living cells to treat or cure a disease.
- **Clinical trial:** research study in which people are assigned to interventions to evaluate the effects on health outcomes.
- **Viral vector:** type of genetically modified virus used in gene therapy to carry a new or corrected gene into a cell.
- **Volume versus value:** volume-based payments are made based on how much of a medicine is prescribed, regardless of how well it works. Value-based payments are contingent upon how well a treatment works for a patient.

drug is paid for based on how much of it is used, regardless of how well the prescription works for a patient. Industry leaders, academic experts, and policymakers are examining innovative payment models to help the system adjust to these new types of therapies, including switching to a value-based payment system.

There are different types of value-based payment models, but they center on a key concept: to pay for a medicine based on its value, or how well it works, instead of simply how much of it is prescribed. One value-based model being explored is an outcomes-based approach where patients and insurers pay for medications or treatments based on how well they work for the patient. For instance, a drug manufacturer would only require payment for their product if the therapy halts the disease's progression or if a patient enters remission within a specified window of time. Because these therapies are so specialized, there is no guarantee they will work for every patient with a specific condition. In this payment model, both drugmakers and payers enter into an agreement knowing payment is only made if the treatment is effective. This incentivizes drugmakers to develop innovative medicine and allows payers to be shielded from financial burden if the treatment fails.

Another value-based model being examined is an installment plan. Under this payment mechanism, insurers would pay drugmakers for a treatment over a defined period of time, potentially several years, similar to how Americans are used to paying for houses or cars. This model would likely also be tied to some sort of metric like the effectiveness of the treatment.

Both models discussed leave questions to be answered, such as what happens if a patient switches insurers? Who handles the rest of the payment to be made? How does CMS navigate value-based plans such as installments when Medicaid is required to get the "best price" for a treatment? Academic institutions like the Duke Margolis Center for Health Policy, the Massachusetts Institute of Technology NEWDIGS program, and the University of Southern California Schaeffer Center for Health Policy are analyzing these issues by bringing together patients, payers, manufacturers, and policymakers to address these concerns.

## Looking Ahead

The FDA expects to see a large increase in the number of gene therapy product approvals over the next several years. Specifically, former Commissioner Scott Gottlieb announced earlier this year that the FDA anticipates receiving more than 200 investigational new drug (IND) applications per year by 2020. This would add to the more than 800 active cell-based or directly administered gene therapy INDs already on file at the FDA. By 2025, they expect to approve 10 to 20 cell and gene therapy products every year based on the current pipeline and success rates of these transformative medicines.

Gene therapy holds incredible promise for patients and families that have run out of options. For some, it can extend a life that was not expected to make it past early childhood, and for others it can restore function to parts of the body they've never been able to use before. However, as these treatments become more available to patients, the U.S. healthcare system will need to adapt. As Congress investigates ways to lower costs in healthcare, it must consider how the healthcare system will adjust to innovative, more specialized, and more expensive medicine while ensuring these potentially life-saving treatments are developed and accessible to patients in need.

- **Outcomes-based payment:** type of value-based payment model where payment is based on the effectiveness of a treatment for a given patient over time.
- **Installment plan:** type of value-based payment model where drugmakers are reimbursed over a defined period of time instead of receiving a lump-sum payment.

A full glossary of common health care terms can be found [HERE](#).

## Additional Resources

[Business Insider - Massachusetts Initiative](#)

[Duke Margolis Center - Consortium to Advance Value-Based Payment](#)

[FDA - Approved Cellular and Gene Therapy Products](#)

[FDA - Cellular and Gene Therapy Products](#)

[FDA - Clinical Research](#)

[FDA - Gottlieb Statement on Policies for Gene Therapies](#)

[FDA - Statement on First Gene Therapy Approval](#)

[FDA - What is gene therapy?](#)

[Fortune - Innovative Pricing](#)

[Medicaid.gov - Drug Rebate Program](#)

[MIT - NEWDIGS Program](#)

[Modern Healthcare - Value-based Contracts](#)

[NCI - Viral Vector](#)

[NIH - How does gene therapy work?](#)

[NPR - First Gene Therapy For Inherited Disease Gets FDA Approval](#)

[USC Schaeffer Center - Redefining Value in Cancer Care](#)