FAST*facts*

AUGUST 2024

GENETICALLY TARGETED TECHNOLOGIES

As medical science advances, new treatments are redefining what's possible for patients, their families and their health care providers. One example is genetically targeted technologies. These medications treat disease by targeting the proteins that cause it. Researchers are discovering that genetically targeted technologies can effectively treat both common diseases and rare conditions.

Q: What are genetically targeted technologies?

Medications using genetically targeted technology work by turning off specific proteins that cause disease. These medications are precise, reducing side effects for patients.

Genetically targeted technologies are generally delivered via injection. Clinicians treat patients once or twice a year in a medical office rather than patients needing to take daily medication at home.

Q: What conditions do genetically targeted technologies treat?

These targeted technologies can treat inherited diseases as well as infectious ones. They offer long-lasting symptom relief for patients with:

- **Rare diseases,** such as amyloidosis
- **Pediatric diseases,** such as spinal muscular atrophy
- **Cardiovascular diseases,** such as familial hypercholesterolemia
- **Neurological diseases,** such as Duchenne muscular dystrophy

The first decade of these treatments has given fresh hope to patients and their families.

Ongoing research suggests that, in the years ahead, genetically targeted technologies could also treat patients with certain autoimmune disorders, cancers and additional cardiovascular diseases, among other conditions.

Q: Are genetically targeted technologies a type of gene therapy?

No. Though the terminology is similar, genetically targeted technologies do not fall under the umbrella of gene therapy.

Gene therapy entails altering a patient's DNA—their genetic code. That typically means modifying or replacing a gene that causes disease.

Medications that use genetically targeted technology work differently. They rely on RNA—a different type of genetic material—to help the body stop producing proteins that cause disease. They do not permanently change a person's genetic make-up.

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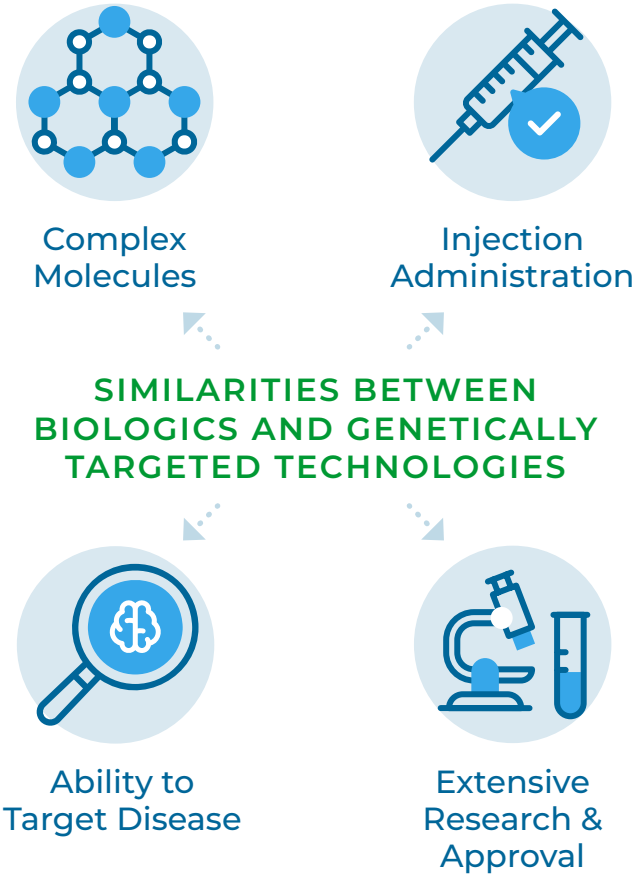
Q: Are genetically targeted technologies the same as biologics?

The two have many similarities. Both biologics and genetically targeted technologies synthesize complex molecules, are typically administered by injection, and allow the body to recognize and stop disease. Moreover, both biologics and genetically targeted technologies require an extensive and costly research and approval process.

The two treatment types are, however, regulated through different pathways under the U.S. Food and Drug Administration. The distinction could have unintended consequences for genetically targeted technologies and the patients who stand to benefit from them.



Q: How can policymakers encourage ongoing research and development?

Developing genetically targeted technologies is a long and costly process. Each treatment that's approved for patients helps to fund the next generation of innovation.

But the cost and timeline of medical innovation has particular significance since Congress passed the Inflation Reduction Act of 2022. Among other provisions, the legislation gives Medicare more control over the price of the drugs it covers under Part B and Part D. Thus, the timeline before potential price negotiation is now a key factor as life sciences companies weigh which novel medications justify ongoing investment.

Biologics have 11 years before becoming eligible for price negotiation. Genetically targeted technologies, despite their similarities to biologics, have only seven.

Policymakers can make a simple adjustment to encourage ongoing research and investment in genetically targeted technologies. Bipartisan legislation called the Maintaining Investments in New Innovation, or MINI, Act would group genetically targeted technologies with biologics for the purposes of Medicare price negotiation. The adjusted timeline could encourage and incentivize ongoing investment in genetically targeted technologies for still more conditions and the patients who live with them.

> Policymakers can make a simple adjustment to **encourage ongoing research and investment.**

CONCLUSION

Genetically targeted technologies have offered patients exciting new treatment options. And they extend the promise of serving even more patient populations in the years to come.

By aligning policy incentives with the needs of patients and their families, Congress can encourage the ongoing research and innovation that serves as a source of hope for patients.

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