Gene editing therapies: driving toward the cure

Innovation at the speed of life™

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Introduction

Beyond treatment and towards cures

There are an estimated 300 million people living with a rare disease globally, and 72% of those rare diseases are caused by a genetic mutation.[1] Patients with these chronic, genetic conditions must often settle for symptom management—taking pills, tolerating injections and enduring surgeries as part of a lifetime of treatment. Furthermore, rare diseases are often overlooked and underfunded, which further complicates care, leaving patients and care teams without the knowledge and resources needed to address the root cause of a condition.

Gene editing has the potential to change that. As our knowledge of human genetics has grown, we've unlocked a brand-new branch of medicine called genomic medicine that may do more than treat symptoms—it could create cures.

The foundation of genomic medicine is sequencing. Next-generation sequencing has made genetic sequencing cheaper, faster, and more prevalent, allowing physicians the opportunity to diagnose, treat and prevent diseases at a molecular level. With the advent of gene editing tools like CRISPR, it's possible to correct errors in a patient's DNA, addressing the root cause of a condition.

The first therapies utilizing gene editing technologies have focused on rare, monogenic diseases, where a single edit can address the problem. For many of these rare conditions, the underlying genetic mutation is already known, providing gene editing therapy developers with a clear target.

By building on these early successes, the potential applications of gene editing medicines can be dramatically expanded.

Advancing gene editing therapies to benefit a wider patient population will therefore require more than just strong science; it will also require strong networks. In order to fulfill the promise of gene editing, we must focus on several key areas.

1. Bring together the top minds from academia, industry and government organizations to establish a clear pathway for gene editing therapies.

2. Accelerate regulatory approval, allowing innovators to rapidly create therapies for the 8,000+ genetic disorders that could benefit from gene editing.

3. Build systems that provide reliable and affordable access to genetic editing for patients around the globe.

Genomic medicine, including gene editing, is an exciting new branch of medicine that requires bold action and collaboration to unlock its full potential. At Danaher, we're committed to solving the hardest problems—together—so these breakthroughs benefit everyone.

1. Nguengang Wakap S, Lambert DM, Olry A, et al. Estimating cumulative point prevalence of rare diseases: analysis of the Orphanet database. European Journal of Human Genetics. 2019;28(2):165-173. doi:10.1038/s41431-019-0508-0

Quantifying patient needs

Rapidly expanding possibilities for genomic medicine

6%

of the global population has a rare disease. They're individually uncommon, but collectively pervasive.[2]

40B

gigabytes of genomic data are generated each year.[3]

$100B

USD projected investment in the genomic market by 2030.[4]

2. Chung CCY, Hong Kong Genome Project, Chu ATW, Chung BHY. Rare disease emerging as a global public health priority. Front Public Health. 2022;10:1028545. doi:10.3389/fpubh.2022.1028545
3. Genomic Data Science Fact Sheet. Accessed July 28, 2025. https://www.genome.gov/about-genomics/fact-sheets/Genomic-Data-Science
4. Genomics Market | USD 176.28 Billion by 2034. Accessed September 30, 2025. https://www.novaoneadvisor.com/report/genomics-market

Connecting key players

Bridging the gaps between discovery and clinical application

Many of the world's most significant scientific breakthroughs and innovations began in academic laboratories. The Nobel Prize-winning discovery of the CRISPR/Cas gene editing system was an academic discovery that changed the gene editing landscape overnight.

But all too often, discoveries occur in silos. Some people worried that CRISPR gene editing would stay restricted to academia, never reaching patients. Fortunately, dedicated leaders from both academia and industry pushed through these barriers to achieve regulatory approval for the initial CRISPR-based therapies. However, if more patients are to benefit from this scientific advancement, we need people and organizations dedicated to discovering and manufacturing these therapies at scale, moving knowledge out of the lab and into a clinical setting.

That's where Danaher comes in. We forge partnerships with academia through our Beacons program.[5] By teaming up with some of the brightest minds and hardest-working labs around the globe, we ensure academic researchers have the support they need to advance new discoveries. Our Beacons aim to improve on critical aspects of gene editing, including harnessing the power of CRISPR to accelerate therapy development or fine-tuning the viral vectors that deliver those gene-editing therapies to target cells.

Through the Beacons program, the knowledge accumulated within academic labs is connected to Danaher's global network of businesses. This is how scientific ideas become real-world solutions. Together, we transform promising technologies into accessible tools for drug discoverers, healthcare providers and patients. Danaher businesses operate across the varied fields of life sciences, biotechnology and diagnostics, sharing a data-driven approach to continuous improvement called the Danaher Business System (DBS). With DBS as a foundation, Danaher businesses and our academic partners innovate together, building on discoveries to create rapid, cost-effective cell and gene therapies.

For example, Danaher businesses recently collaborated with doctors at the Children's Hospital of Philadelphia and the University of Pennsylvania to develop the world's first personalized gene-editing therapy in just six months – a process that typically takes 18-24 months. To design, manufacture and receive regulatory approval for this custom CRISPR therapy in record time required precise coordination between academia, industry and government organizations. By bridging the gap between these key players, Danaher helped to better integrate the full drug discovery and development lifecycle.

Scientific breakthroughs only happen when knowledge is shared—and when the toughest challenges are tackled together. By strengthening the collaborative networks that drive scientific innovations, Danaher is laying the groundwork for a future where genomic medicine is available to everyone who needs it.

5. https://www.danaher.com/danaher-beacons

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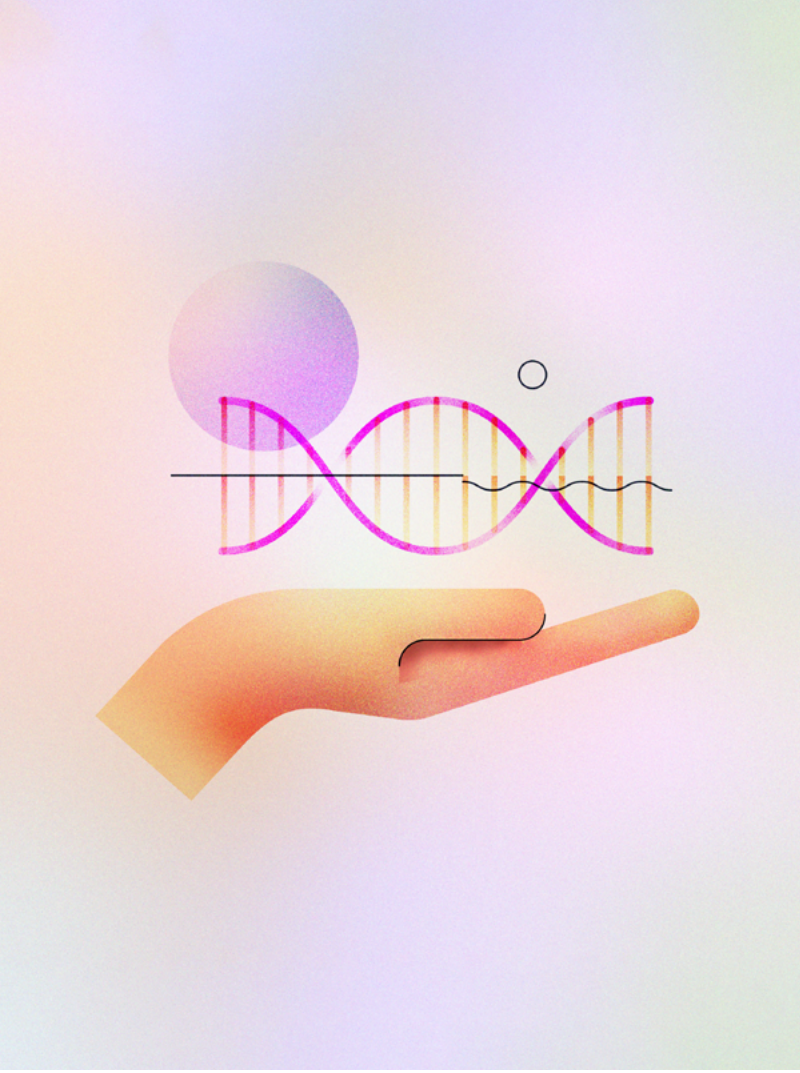
The science is there. The tools are there. But the ability to execute on the promise of genomic medicine requires collaboration across multiple partners. Danaher is working at the center, connecting the central nodes of this ecosystem to make gene editing a reality."

Sadik Kassim, PhD
Chief Science Officer & Chief Technology Officer, Danaher Omic Solutions and Genomic Medicines

Developing a modular toolkit

Gene editing templates



The current FDA model for approval of new medicines was not designed with gene editing therapies in mind. For more traditional modalities, the exact same drug is given to every patient with a given condition, making it easy to design robust clinical trials. But gene editing therapies are carefully designed to correct genetic errors that may be unique to a single patient. The traditional regulatory process is simply too rigid and time-consuming to accommodate the more individualized nature of these gene therapies. This has made the risks and costs of pursuing gene therapies relatively high and contributed to the slow development of gene editing technologies. This is especially true for rare disorders, where the patient population is small and the odds of seeing a good return on investment may be low.

Danaher is looking to change this. Working with the Innovative Genomics Institute (IGI) and the FDA, Danaher is pursuing a new regulatory pathway that would approve gene editing therapies based on a platform designation. The rationale for this approach is that gene editing platforms, such as CRISPR, are made of discrete building blocks. These components include the gene editing protein, the guide RNA, and the delivery vehicle used to bring the editor and guide components to the right cells. In many cases, the editors and delivery vehicles remain relatively unchanged regardless of the disease they are targeting. For example, the CRISPR platform combines standardized elements with the personalized guide RNAs that determine the specificity of the therapy.

Danaher sees an opportunity to build an indication-agnostic toolkit, filled with these gene editing building blocks. In collaboration with IGI, we're developing a modular, plug-and-play framework in which doctors can select CRISPR editors and delivery vehicles that have been optimized for safety, quality and manufacturing consistency. When paired with a personalized guide RNA, these therapies could address patient-specific mutations.

This platform will greatly accelerate regulatory approval. IDT, a Danaher business, is going a step further by enhancing the design of these CRISPR components using artificial intelligence (AI) so that therapies designed with our platform are not only approved faster, but they will also have increased accuracy and editing efficiency. With these efforts, Danaher is providing therapeutic developers with the tools they need to rapidly develop gene editing therapies.

We envision a world where every genetic disease is treatable, and the CRISPR toolkit is our first step toward that future. The journey to an approved gene editing therapy is long and complex, but given the right resources and information, we know scientific innovators can transform the lives of millions of patients.

Overcoming barriers to access

Making gene editing therapies more accessible

Developing and manufacturing gene editing therapies is only part of the picture. As Danaher empowers developers to build gene editing therapies, we need to make sure these cures can get to the patients who need them. We're focusing on effective and equitable distribution of gene editing therapies, making this novel class of treatment available to everyone who needs it.

As part of Danaher's work on this important challenge, the Advanced Research Projects Agency for Health (ARPA-H) has awarded Cytiva (Global Life Sciences Solutions USA, LLC), a Danaher company, an Other Transaction (OT) as part of the ARPA-H Engineering of Immune Cells Inside the Body (EMBODY) program that focuses on decreasing the costs of CAR-T therapy for solid tumors to a team led by Danaher Omics Solutions and Genomic Medicines CTO, Dr. Sadik Kassim, along with industry and academic partners.[6] CAR-T therapy is a category of gene edited cell therapy where immune cells are collected from a patient, genetically manipulated in a lab, and then reintroduced to the patient as a form of cancer therapy. However, the complexity of these steps and the logistics involved drives production costs up and limits the scalability and accessibility of this approach.

This ARPA-H OT is designated to build transformative in vivo CAR-T therapies for solid tumors. At the moment, developing a CAR-T therapy costs several hundred thousand dollars. This work will focus on enabling a cost-sensitive patient-centered experience, with a desired goal of shifting from complex inpatient cell harvest and protracted CAR-T generation waiting period to a simple injection on-demand at local points-of-care that enables access and scale-out of immunotherapies across the healthcare system. These efforts will not only advance CAR-T therapies but also provide insights into how to make other gene editing therapies more cost-effective as well.

This project represents an exciting opportunity for leaders across industry to come together and solve some of the hardest problems in scaling gene editing therapies. By addressing the above problem, Danaher leaders are helping build a future where life-changing medicine is available to all who need it.

Conclusion

Gene editing therapies: a game-changer

Gene editing is fundamentally shifting how we think about medicine. Instead of focusing on the symptoms, gene editing therapies allow physicians to address the source of the problem. That's the goal we're focused on, and achieving this goal will take teamwork. We won't get there overnight, but at Danaher, we understand what's needed to fuel innovation that changes lives. We're building the expertise, connections and platforms developers need to accelerate the entire pipeline of gene editing therapies. **We see a path to a healthier future, and we're driving the innovation to deliver it.**

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