

# Genetic and AI Drive Precision Medicine

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**Received:** 01-Jul-2025, Manuscript No. jowt-25-174556; **Editor assigned:** 03-Jul-2025, PreQC No. jowt-25-174556(PQ); **Reviewed:** 17-Jul-2025, QC No. jowt-25-174556; **Revised:** 22-Jul-2025, Manuscript No. jowt-25-174556(R); **Published:** 29-Jul-2025, **DOI:** 10.4172/2165-7904.1000817

**Citation:** Collins A (2025) Genetic and AI Drive Precision Medicine. jowt 15: 817.

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## Abstract

Recent advancements in biotechnology are transforming healthcare and industrial processes. *Gene editing techniques like base editing and prime editing offer precise modifications for conditions such as hypercholesterolemia and various genetic disorders. Coupled with Artificial Intelligence (AI) and next-generation sequencing, precision medicine is achieving new heights in cancer and cardiovascular disease treatment. Nanotechnology enhances gene therapy delivery, while synthetic biology and omics technologies accelerate drug discovery and sustainable product synthesis. This collective progress signifies a paradigm shift towards personalized, efficient, and transformative solutions in biological and medical fields.*

## Keywords

Gene editing; CRISPR/Cas9; Prime editing; Next-generation sequencing; Artificial Intelligence (AI); Precision medicine; Nanotechnology; Omics technologies; Synthetic biology; Gene therapy; Cardiovascular diseases; Cancer treatment; Metabolic engineering

## Introduction

The landscape of modern biomedical research and technology is undergoing a profound transformation, driven by breakthroughs in genetic engineering, computational methods, and advanced delivery systems. These developments are not just incremental; they represent a paradigm shift towards more precise, personalized, and effective interventions across a spectrum of diseases and industrial applications. At the forefront of this revolution are sophisticated gene editing techniques, which are redefining the boundaries of what is treatable and achievable in medicine.

A significant leap forward in therapeutic gene editing involves

in vivo base editing, a method that precisely targets and modifies single bases within the genome. This approach has shown remarkable success in lowering LDL cholesterol in macaques by specifically targeting the PCSK9 gene, moving beyond traditional gene knockout methods to offer a refined strategy for hypercholesterolemia treatment. What this really means is a promising new avenue for metabolic disease therapies [1].

Further expanding the toolkit of genetic modification is prime editing, a revolutionary technique that facilitates precise insertions, deletions, and all twelve possible base-to-base conversions. Importantly, it achieves these modifications without necessitating double-strand breaks or the use of donor DNA, addressing key limitations of earlier methods. This advancement drastically broadens the scope of treatable genetic disorders, marking a crucial step towards more accurate and versatile genome engineering [2].

Similarly, CRISPR/Cas9 technology continues to demonstrate its transformative potential, particularly in the realm of cardiovascular diseases. This powerful gene-editing system offers the ability to correct genetic defects implicated in conditions such as hyper-

trophic cardiomyopathy, paving the way for new therapeutic strategies that can profoundly impact heart health through precise genetic modifications [3].

The diagnostic and prognostic capabilities in medicine have also seen a substantial upgrade with the widespread adoption of next-generation sequencing (NGS). In oncology, for example, NGS is instrumental for accurate cancer diagnosis, predicting disease progression, and guiding personalized treatment plans. It identifies specific genetic mutations within tumors, allowing for highly targeted therapies and ultimately leading to better patient outcomes based on their individual molecular profiles. This technology is fundamentally transforming cancer care [4].

Beyond human health, the convergence of synthetic biology and metabolic engineering is reshaping the production of valuable natural products. Scientists are now designing and optimizing microbial cell factories to synthesize a diverse range of compounds, from pharmaceuticals to biofuels, with greater efficiency and sustainability. Genetic manipulation is central to unlocking new biosynthetic pathways and scaling up production, offering a truly transformative approach to industrial biotechnology [5].

The integration of Artificial Intelligence (AI) with precision medicine is another cornerstone of this new era. AI algorithms are proving invaluable in processing vast amounts of genetic and clinical data, enabling the identification of complex disease patterns, accurate prediction of disease risk, and the optimization of treatment plans tailored to an individual's unique genetic makeup. This synergy between AI and genomics is dramatically improving healthcare, paving the way for far more personalized and effective medical interventions [6].

In the realm of specific disease targets, gene therapy is fundamentally changing the landscape for inherited retinal diseases. Recent advancements showcase how targeted genetic interventions are effectively restoring vision or preventing its loss in patients with conditions previously considered untreatable. This work underscores the real-world impact of genetic therapies in transforming lives and offering hope where little existed before [7].

Concurrently, significant strides in genetic engineering are profoundly altering the approach to neurological disorders. Novel techniques like CRISPR and various gene therapy modalities are being applied to conditions such as Huntington's and Parkinson's disease, with a focus on correcting the underlying genetic causes rather than merely managing symptoms. This shifts the potential for treatment from palliative care to direct disease modification [8].

Moreover, nanotechnology is dramatically enhancing the deliv-

ery and efficacy of gene therapy, especially in cancer treatment. Nanoparticles are engineered to precisely deliver genetic material directly to tumor cells, effectively overcoming barriers that traditional delivery methods often encounter. This represents a game-changer for targeted therapies, showcasing how interdisciplinary approaches are making gene-based cancer treatments more effective and less toxic [9].

Finally, the powerful combination of omics technologies, such as genomics and proteomics, with advanced computational methods is fundamentally reshaping drug discovery. These integrated tools enable the identification of novel drug targets, predict drug efficacy, and facilitate the personalization of treatments based on an individual's comprehensive molecular profile. This marks a significant shift in pharmaceutical research, with genetics and big data analytics transforming the entire drug development pipeline [10].

## Description

The recent surge in biotechnological advancements is ushering in an era of unprecedented precision and effectiveness in treating diseases and developing sustainable industrial solutions. A central theme is the refinement of gene editing techniques. For instance, in vivo base editing represents a significant advancement by enabling precise single-base modifications, effectively lowering LDL cholesterol in macaques through targeted editing of the PCSK9 gene. This approach offers a promising alternative to traditional gene knock-out methods for hypercholesterolemia, illustrating a move towards more nuanced genetic therapies for metabolic diseases [1]. What this means is that we're moving beyond blunt tools to highly specific interventions.

Adding to the sophistication of gene editing, prime editing emerges as a revolutionary technique, facilitating precise insertions, deletions, and all twelve possible base-to-base conversions. This is achieved without the need for double-strand breaks or donor DNA, which historically posed challenges for gene-editing safety and versatility. The capacity of prime editing to perform such a broad range of modifications greatly expands the potential to treat a wide spectrum of genetic disorders, marking a crucial step towards more accurate and versatile genome engineering [2]. Concurrently, the well-established CRISPR/Cas9 technology continues to find new applications, particularly in the critical area of cardiovascular diseases. Research highlights its potential in correcting specific genetic defects that cause conditions like hypertrophic cardiomyopathy, thereby fostering the development of innovative therapeutic strategies for heart health. The transformative impact of such pre-

cise gene editing on cardiac care opens doors for highly effective and personalized treatments [3].

Beyond direct genetic modification, diagnostic and therapeutic strategies are evolving rapidly. Next-generation sequencing (NGS) has become indispensable in oncology, transforming cancer care through its ability to provide precise diagnoses, prognoses, and to guide personalized treatment strategies. By identifying specific genetic mutations unique to individual tumors, NGS facilitates the selection of highly targeted therapies, leading to significantly better patient outcomes. This genomic approach is truly reshaping how we understand and combat cancer [4]. Furthermore, the power of Artificial Intelligence (AI) is being harnessed to advance precision medicine, especially in synthesizing vast quantities of genetic and clinical data. AI algorithms are adept at identifying complex patterns, predicting disease risk, and optimizing treatment plans tailored to an individual's unique genetic makeup. This integration of AI with genomics is poised to revolutionize healthcare, making medical interventions far more personalized and effective [6].

The impact of these technologies extends beyond human health to industrial applications and novel drug discovery. Synthetic biology and metabolic engineering are converging to reshape the production of natural products. Scientists are now able to design and optimize microbial cell factories to synthesize valuable compounds, ranging from pharmaceuticals to biofuels, with unprecedented efficiency and sustainability. Genetic manipulation is central to unlocking new biosynthetic pathways and scaling up industrial production, offering a truly transformative approach to biotechnology [5]. Meanwhile, gene therapy itself continues to make monumental strides, particularly in treating inherited retinal diseases. Recent reviews detail how targeted genetic interventions are successfully restoring vision or preventing its loss in patients suffering from previously untreatable conditions, showcasing the profound life-changing potential of genetic advancements [7]. Similarly, genetic engineering is profoundly altering our approach to neurological disorders. Novel techniques like CRISPR and advanced gene therapy applications are being explored for conditions such as Huntington's and Parkinson's disease, focusing on correcting the underlying genetic causes rather than just managing symptoms, thus moving towards direct disease modification [8].

To enhance the efficacy of these genetic interventions, nanotechnology plays a crucial role, especially in gene therapy for cancer treatment. Nanoparticles are being engineered for precise delivery of genetic material directly to tumor cells, effectively overcoming the physiological barriers that often limit traditional therapeutic approaches. This advancement is a game-changer for targeted ther-

apies, illustrating how interdisciplinary research is making gene-based cancer treatments both more effective and less toxic [9]. Finally, the synergistic application of omics technologies, including genomics and proteomics, with advanced computational methods is fundamentally reshaping the entire drug discovery pipeline. These integrated tools enable the identification of novel drug targets, accurately predict drug efficacy, and facilitate the personalization of treatments based on an individual's comprehensive molecular profile. This represents a significant shift, with genetics and big data analytics transforming pharmaceutical research from its foundational stages [10].

## Conclusion

Recent research highlights the rapid evolution of genetic and computational technologies across various scientific and medical fields. Innovations in gene editing, including in vivo base editing for conditions like hypercholesterolemia, demonstrate a move towards highly precise single-base modifications, surpassing traditional gene knockout methods. Prime editing further expands this capability, allowing for precise insertions, deletions, and all 12 base-to-base conversions without harmful double-strand breaks. Such advancements are crucial for treating a wide array of genetic disorders.

CRISPR/Cas9 technology is now actively applied to cardiovascular diseases, offering the potential to correct genetic defects and develop new therapeutic strategies for heart health. In oncology, next-generation sequencing is transforming cancer care by enabling precise diagnosis, prognosis, and personalized treatment plans based on individual tumor profiles.

Beyond direct medical applications, synthetic biology and metabolic engineering are redefining natural product synthesis, using optimized microbial cell factories for compounds like pharmaceuticals and biofuels. The integration of Artificial Intelligence (AI) with precision medicine is enhancing healthcare by processing vast genetic and clinical data, predicting disease risks, and tailoring treatments to individual genetic makeups.

Gene therapy is fundamentally changing the landscape for inherited retinal diseases and neurological disorders, offering hope for vision restoration and direct disease modification in conditions like Huntington's and Parkinson's. Nanotechnology plays a vital role in improving gene therapy delivery for cancer, while omics technologies combined with computational methods are accelerating drug discovery by identifying new targets and personalizing treatments. This comprehensive overview shows a future where ad-

vanced biotechnologies are creating more effective, targeted, and personalized solutions in medicine and industry.

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