OPEN ACCESS Freely available online

Current Synthetic and Systems Biology

Commentary

Regulatory and Safety Considerations in the Era of Widespread Genome Editing

Isabella Hayes*

Department of Medical Genome Science, University Hospital Rostock, Rostock, Germany,

DESCRIPTION

Genome editing has emerged as one of the most revolutionary technological advancements in the life sciences, fundamentally transforming how researchers study, manipulate and understand biological systems. At its core, genome editing refers to the precise modification of an organism's DNA sequence, enabling targeted alterations that were previously impossible or impractical. From correcting disease causing mutations to engineering plants and microbes for enhanced productivity, genome editing has unlocked unprecedented opportunities across biology, medicine, agriculture and biotechnology.

When scientists first demonstrated the ability to insert or delete genes in bacterial and eukaryotic cells. Early methods relied on homologous recombination or random mutagenesis, which, were limited by low efficiency and a lack of precise control. The advent of site specific nucleases, such as Zinc Finger Nucleases (ZFNs) and Transcription Activator Like Effector Nucleases (TALENs), represented a major leap forward by enabling researchers to target specific genomic loci with greater precision. These engineered nucleases function by recognizing unique DNA sequences and inducing double stranded breaks, which are then repaired by the cell's natural DNA repair machinery, resulting in targeted modifications.

The real paradigm shift in genome editing came with the discovery and rapid adoption of the CRISPR Cas system. Originally identified as an adaptive immune mechanism in bacteria and archaea, CRISPR Cas (Clustered Regularly Interspaced Short Palindromic Repeats) has become the most widely used genome editing platform due to its simplicity, versatility and efficiency. In its most common form, CRISPR Cas9 uses a programmable guide RNA to direct the Cas9 nuclease to a complementary DNA sequence, where it induces a double-stranded break. This break can then be repaired by Non Homologous End Joining (NHEJ), which often results in small insertions or deletions, or by homology directed repair (HDR) if a repair template is provided, enabling precise sequence replacement. Variants of CRISPR, such as Cas12 and Cas13, have expanded the toolbox further, allowing not only DNA editing but also RNA targeting, base editing and epigenetic modulation.

One of the most remarkable features of genome editing is its versatility across organisms and cell types. CRISPR and related technologies have been successfully applied in bacteria, yeast, plants, animals, and human cells. In agriculture, genome editing allows the rapid development of crop varieties with improved yield, disease resistance, drought tolerance, and nutritional content without introducing foreign DNA, distinguishing it from traditional Genetically Modified Organisms (GMOs). In microbial biotechnology, engineered strains can be optimized for biofuel production, pharmaceutical synthesis, and environmental remediation. In biomedical research, genome editing facilitates the creation of model organisms with disease specific mutations, enabling the study of complex genetic disorders and accelerating drug discovery.

In human medicine, genome editing holds extraordinary promise for treating genetic diseases at their source. Monogenic disorders such as sickle cell disease, beta thalassemia, cystic fibrosis, and Duchenne muscular dystrophy are prime targets for CRISPR mediated correction. *Ex vivo* editing approaches, where patient derived cells are modified outside the body and then reintroduced, have already shown remarkable success in clinical trials. For example, hematopoietic stem cells from patients with sickle cell disease have been edited to reactivate fetal hemoglobin production, reducing disease symptoms and improving quality of life. The potential for *in vivo* editing, where CRISPR components are delivered directly into tissues, is also rapidly advancing, opening the possibility of treating a broader range of diseases, including metabolic disorders, viral infections and even certain cancers.

Correspondence to: Isabella Hayes, Department of Medical Genome Science, University Hospital Rostock, Rostock, Germany, E-mail: isabella.hayes@gmail.com

Received: 02-Jun-2025, Manuscript No. CSSB-25-39254; **Editor assigned:** 04-Jun-2025, PreQC No. CSSB-25-39254 (PQ); **Reviewed:** 17-Jun-2025, QC No. CSSB-25-39254; **Revised:** 24-Jun-2025, Manuscript No. CSSB-25-39254 (R); **Published:** 01-Jul-2025, DOI: 10.35248/2332-0737.25.13.110

Citation: Hayes I (2025). Regulatory and Safety Considerations in the Era of Widespread Genome Editing. J Curr Synth Syst Bio. 13:110.