PUBLIC HEALTH AND GENE THERAPY TECHNOLOGY

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ABSTRACT:

In the field of gene therapy, the convergence of public health and technology offers a revolutionary new direction for medical research. Advances in technology, such CRISPR-Cas9 and other tools for gene editing, have completely changed the feasibility and accuracy of gene therapies. A thorough public health framework that addresses accessibility, moral issues, and societal ramifications is required in light of this change.Ensuring that gene therapies are distributed fairly, reducing inequities, and maximizing their integration into current healthcare systems are all made possible by public health activities. Informed consent, long-term effects, and ethical issues related to gene editing highlight the significance of public health recommendations.In the rapidly changing field of gene therapy, this abstract emphasizes the underscoring the necessity of cooperative efforts to capitalize on these breakthroughs.By altering how a person's genes are expressed, gene therapies can be used to treat, prevent, or even cure a disease. They are a novel approach to treating hereditary illnesses, but the market for them and the healthcare system are still developing. There are noticeable variations between health technology assessments (HTAs) conducted in different nations because health technology assessment (HTA) organizations have not yet developed a standardized methodology for evaluating gene treatments.

KEYWORDS:

Genome Editing, CRISPR-Cas9, Lentiviruses, Immunotherapy, Public Health and Gene Therapy, Inherited Genetic Disorders, Health Technology Assessment.

INTRODUCTION:

A novel approach to treat, cure, or ultimately prevent disease by changing a person's gene expression" is how the American Medical Association describes gene therapies.

Although these therapies are a novel approach to treating genetic disorders, including uncommon and complex diseases, they are still in the early stages of development both in the healthcare system and on the market.Gene therapy stands at the forefront of transformative medical interventions, harnessing the power of molecular and genetic advancements to address a myriad of diseases at their core. This innovative field revolves around the deliberate modification of an individual's genes to treat or prevent disorders, presenting a paradigm shift from traditional therapeutic approaches. With the evolution of cutting-edge technologies like CRISPR-Cas9, AAVs, and lentiviruses, the precision and feasibility of gene therapy have reached unprecedented levels. This introduction explores the diverse technologies employed, ethical considerations, and the crucial interplay between public health and the implementation of gene therapy..Gene therapy attempts to restore the normal functioning of cells and tissues in a patient by changing, repairing, or replacing damaged genes in their body. (1).Public health is a dynamic field dedicated to safeguarding and improving the well-being of communities. As we delve into the 21st century, the intersection of public health and gene therapy technology presents a groundbreaking frontier. Gene therapy, a revolutionary approach aimed at treating or preventing diseases by modifying the genetic makeup of individuals, holds immense promise for transforming healthcare outcomes.Join me on this exploration of the symbiotic relationship between public health and the transformative potential of gene therapy technology. We are setting out on a path toward customized therapies, focused interventions, and the possible reinterpretation of disease prevention tactics as we investigate the potential synergies between gene therapy and public health. Come explore the developments and effects of this state-of-the-art technology on how healthcare will evolve in the future.

Gene therapy encompasses a spectrum of strategies aimed at correcting, replacing, or regulating genes to treat or prevent diseases. Here's a more detailed exploration:

1. TECHNOLOGICAL LANDSCAPE:

- CRISPR-Cas9:Known for its accuracy, efficiency, and versatility in gene editing, CRISPR-Cas9 allows researchers to precisely modify DNA sequences.

- Viral Vectors:AAVs and lentiviruses are commonly used as carriers to deliver therapeutic genes into target cells. They efficiently transport genetic material without causing disease.

2. APPLICATIONS:

- Monogenic Disorders: Gene therapy shows promise in treating diseases caused by a single gene mutation, such as cystic fibrosis and sickle cell anemia.

- Cancer:Immunotherapy, a form of gene therapy, enhances the immune system's ability to target and destroy cancer cells.

3. Challenges and Ethical Considerations:

- Off-Target Effects:Ensuring the precision and safety of gene-editing tools remains a challenge.

- Informed Consent:The ethical implications of altering the human genome raise questions about informed consent and long-term consequences.

4. PUBLIC HEALTH INTEGRATION:

- Equitable Access: Public health initiatives play a crucial role in ensuring widespread and equitable access to gene therapies.

- Regulatory Frameworks: Establishing ethical and legal frameworks is essential to guide the responsible development and implementation of gene therapy.

5. FUTURE PROSPECTS:

- Personalized Medicine:Gene therapy paves the way for personalized treatments tailored to an individual's genetic makeup.

- Emerging Technologies: Ongoing research continues to introduce new gene-editing tools and delivery methods, expanding the therapeutic potential.

As gene therapy navigates the frontier of medical innovation, its integration with public health measures and ethical considerations will shape its trajectory, influencing how we combat diseases at their genetic roots.(2,3).

GENE THERAPY DRUGS:

Currently, the majority of conventional drug targets are proteins, like enzyme inhibitors and antibodies, that are expressed by genes that cause disease. On the other hand, gene therapy medications hold a broad position in clinical treatment because they have the ability to modify gene transcription and translation processes at the DNA or mRNA level. Several of the most extensively researched gene therapy medications will be covered in this section, including plasmid DNA (pDNA), siRNA, miRNA, ASO, CRISPR/Cas9, and similar ones. A novel technique called RNA interference (RNAi) has emerged recently that has great promise for gene therapy since it can prevent the expression of particular genes.(4)

MICRO RNA:

MiRNA, a small non-coding RNA with an approximate length of 19–22 nt, is encoded by eukaryotic organisms' cell genomes. By encouraging the breakdown of target mRNA or preventing mRNA translation, it controls gene expression, which in turn impacts cell growth, division, apoptosis, and development (5). The mechanism of miRNA is to form an asymmetric RISC by combining with the miRNA-induced silencing complex (miRISC) on the target mRNA. Research has demonstrated that miRNA binds to

miRISC under the action of RISC endonuclease, recognizing the 3′ untranslated region (3′UTRs) of the target mRNA and preventing its translation (6).

CRISPR-COS-9:

Based on plasmid, RNA, and protein, the CRISPR/Cas9 system is currently delivered at three different levels. Delivery of mRNA encoding Cas9 protein has a lower off-target rate than delivery plasmid; direct delivery of Cas9 protein has low immunogenicity; delivery of plasmids encoding Cas9 protein and sgRNA requires fewer transfections, making it convenient and stable. The low cost and ease of use of the pDNA-based CRISPR/Cas9 system have made it a very appealing tool. (7).However, the transcription process and the size of the encoded pDNA will severely impede system delivery and lower gene editing efficiency. The most effective method for using the CRISPR/Cas9 system is to deliver sgRNA and Cas9 protein directly, bypassing pDNA or mRNA expression.Non-viral vectors of the CRISPR/Cas9 system have been successfully delivered to cells and tissues in vivo and in vitro thanks to advancements in nanotechnology in recent years. Mout et al. successfully delivered RNP to the cytoplasm by co-delivering the Cas9 protein and sgRNA using arginine gold nanoparticles (ArgNPs). After that, it was moved to the nucleus, where it was able to edit genes 30% of the time and deliver information up to 90% of the time.(8).

OTHER GENE THERAPY DRUG:

The hairpin structure of shRNA is formed by a stem-loop sequence located in the middle of two short inverted repeats. Through virus-mediated transduction, shRNA maintains its stability with a comparatively high viral infection efficiency. The cell's Dicer enzyme is used to initiate nucleus-wide gene silencing following transduction. In contrast to siRNA, it has a longer-lasting and more potent curative effect, but its transfection efficiency is lower and its preparation takes longer. Zhang et al. treated pancreatic fibrosis by inducing pancreatic stellate cells' mitochondrial apoptosis and delivering TLR4 shRNA (VA-lip-shRNA-TLR4) via cationic liposomes (9)There were also worries that governments might restrict people's or parents' freedom of choice and force them to receive cell and gene therapies if they become widely accessibleThe significance of gene therapy lies in its potential to revolutionize medical treatments by addressing the root causes of diseases at the genetic level. Here are key aspects that highlight its importance:Public Health Impact:Reducing Disease Burden:Successful implementation of gene therapy can contribute to reducing the overall burden of diseases on public health, improving the well-being of

populations(10).The highest levels of support for extending life span, enhancing intelligence, and enhancing strength and fitness were expressed by those who supported the use of gene editing for enhancement purposes(11). According to reports, the Chinese population has a neutral stance towards the use of gene editing for enhancement(12). It was noted that those who had children were more likely to be in support34. Remarkably, a study found that roughly 60% of participants were in favor of using embryonic stem

cells—as long as stringent legal restrictions were in place. Religion's impact on popular beliefs was largely equivocal(11).

HEALTH AND TECHNOLOGY ASSESSMENT (HATS) FOR GENE THERAPY:

Many of the evaluation issues associated with cell and gene therapy apply to rare diseases more generally, and the majority of current cell and gene therapies are designed to treat rare diseases. In the case of cell and gene therapies, however, the challenges are compounded by the potentially transformative nature of the health benefits and the potential long-term nature of the "cure" with significant uncertainty. HTA organs respond to assessment problems arising from cell and gene therapy have received relatively little attention. HTA methods rely in part on currently available evidence, and new methods often require new types of evidence. For example, clinical evidence for cell and gene therapies may be scarce compared to most drugs, and the economic benefits of cell and gene therapies may require additional evidence to demonstrate them and many innovative payment models. the proposal may require additional evidence after the product is placed on the market. In addition, although some publications in the current literature call for a different approach to HTA for cell and gene therapy, most HTA institutions prefer to use a standardized approach for each health technology. (13)

CONCLUSION:

In conclusion, the intersection of public health and gene therapy technology holds immense promise for transforming healthcare. As we navigate the complexities of genetic interventions, it becomes evident that technological advancements, such as CRISPR-Cas9 and viral vectors, empower precise and targeted treatments. Public health plays a pivotal role in ensuring the equitable distribution of these innovations, addressing ethical considerations, and establishing robust regulatory frameworks.The significance of gene therapy lies not only in the potential to cure genetic disorders but also in paving the way for personalized and efficient medical interventions. The collaboration between technology experts, healthcare professionals, policymakers, and the public is crucial for responsible research, implementation, and addressing societal implications.As we move forward, the continuous evolution of gene therapy technologies, coupled with a commitment to ethical practices and public health principles, will shape a future where genetic interventions contribute significantly to improving health outcomes and fostering a more equitable healthcare landscape.In conclusion, the marriage of public health and gene therapy technology represents a transformative frontier in medicine. The precision and potential of gene therapy technologies, including CRISPR-Cas9 and viral

vectors, offer unprecedented opportunities for treating genetic disorders at their root. The collaboration between technological innovation and public health initiatives is essential for harnessing these breakthroughs responsibly.Public health plays a critical role in ensuring that the benefits of gene therapy are accessible to all, addressing issues of equity, affordability, and ethical considerations. Regulatory frameworks and ethical guidelines serve as the cornerstone, guiding the integration of gene therapy into mainstream healthcare.The dynamic interplay between technological advancements and public health strategies underscores the importance of a multidisciplinary approach. As gene therapy continues to evolve, it holds promise not only for monogenic diseases but also for complex conditions like cancer, ushering in an era of personalized and precise medicine.However, challenges persist, including the need for ongoing research, addressing potential long-term effects, and ensuring global access. By fostering collaboration, maintaining ethical standards, and integrating gene therapy into comprehensive public health strategies, we can unlock the full potential of these technologies, paving the way for a healthier and more equitable future.

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