Technologies and Therapy: Genetic Engineering Technologies for Human Gene Therapy

Abstract

In the 1960s, the initial concepts of gene therapy were introduced. More than 1900 clinical trials for the treatment of genetic diseases and cancers, primarily with viral vectors, have been conducted since the early 1990s. Although a variety of treatments for malignant gliomas have been tried, it has been hard to get at invasive glioma cells. For the purpose of delivering genes to invasive gliomas, immortalized neural stem cells (NSC) and a nonlytic, amphotropic retroviral replicating vector (RRV) have attracted attention. Technology for editing the genome that targets insertions at specific sites has developed recently; CRISPR/Cas9, or clustered regularly interspaced palindromic repeats and CRISPR-associated-9, is a particular example of this. More than 30 clinical trials using genome editing technologies have been conducted since 2015, and the outcomes have demonstrated the potential to improve patient outcomes. It is anticipated that gene therapy using CRISPR technologies to treat a wide range of diseases will continue to advance.

Natural food preservatives known as essential oils (EOs) may impair the sensory qualities of foods. Microorganisms can be inactivated by emerging technologies (ETs), but the process may compromise quality parameters at high intensities. The mechanisms of microbial inactivation in combined processes are presented as well as the use of EOs and ETs in this manuscript. Additionally, the benefits, drawbacks, and limitations of EO and ETs were discussed. Lemongrass, lavender, thyme, sweet basil, lime, oregano, mentha, cinnamon, citral, caracole, carbon, geranium, eugenol, citrus extract, mandarin, rosemary, and clove essential oils were found to be effective in inactivating pathogens, spoilage bacteria, and molds when they were combined with cold plasma, ultrasound, irradiation, ultraviolet light, high hydrostatic processing, pulsed light, pulsed electric fields Meat, fish, eggs, milk, plant-based products, and food container surfaces are among the food matrices investigated for EO and ET.

Keywords: Microbial inactivation • Non-thermal technologies • Synergism • Ohmic heating • Sustainable food consumption • Gene therapy • Genome editing • ZFN

Introduction

The first ideas for gene therapy were introduced in the 1960s. Since the beginning of the 1990s, more than 1900 clinical trials for the treatment of genetic diseases and cancers, primarily using viral vectors, have been conducted. Although a variety of treatments for malignant gliomas have been tried, getting at invasive glioma cells has been difficult. Immortalized neural stem cells (NSC) and a nonlytic, amphotropic retroviral replicating vector (RRV) has attracted attention for the purpose of delivering genes to invasive gliomas. Recently, technology has emerged for editing the genome to target insertions at specific locations; one particular illustration of this is CRISPR/Cas9, or clustered regularly interspaced palindromic repeats and CRISPR-associated-9. Since 2015, more than 30 clinical trials utilizing genome editing technologies have demonstrated the potential to improve patient outcomes. Gene therapy using CRISPR technologies to treat a wide range of diseases is expected to progress further [1].

Essential oils (EOs), which are natural food preservers, have the potential to impair

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Development of viral vectors

In the production of cell and gene therapy, viral vectors remained essential components. Numerous genetic conditions, such as Leber's congenital amaurosis (LCA) and reverses lipoprotein lipase deficiency (LPLD), were treated with adeno-associated viral (AAV) vectors. LCA type II achieved remarkable success in phases I/II clinical trials in 2008. Mutations in the RPE65 gene which encodes retinoid isomerase and is highly expressed in the retinal pigment epithelium cause LCA, a rare hereditary disorder of retinal degeneration. These studies demonstrate that recombinant AAV2/2 vectors can deliver RPE65 into retinal pigment epithelial cells and deliver clinical benefits without causing side effects. For patients with LCA type II, the FDA recently granted approval for voretigene neparvovec-rzyl. In 2012, Alipogene tiparvovec Glybera became the first gene therapy-based treatment for LPLD to receive approval in Europe. An intact LPL gene is delivered to the muscle cells by the AAV1 vector. AAV vectors have been used in more than 200 clinical trials for a number of genetic diseases, including haemophilia, retinal dystrophy, and spinal muscular atrophy [4].

One of the most common approaches to gene therapy is still retrovirus. The FDA-approved drug Strimvelis (GlaxoSmithKline, London, UK) is an autologous CD34 (+)-enriched cell population with a gamma retrovirus containing the ADA gene. It was the first exvivo stem cell gene therapy for patients with

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SCID who lacked the ADA gene. As a result, retroviral vectors were frequently utilized to treat other genetic diseases, such as X-SCID. The human immunodeficiency virus (HIV), which infects host cells by inserting DNA into their genome, is the cause of acquired immunodeficiency syndrome, which is caused by the lent virus. Non-dividing cells can be infected by the lent virus; As a result, there are more applications for it. A lent viral vector lacking the peroxisomal adenosine triphosphate-binding cassette D1 was used to treat X-linked adrenoleukodystrophy patients successfully. Oncogene activation was not observed despite the use of a lent viral vector with an internal viral long terminal repeat [5-8].

Conclusion

Even though genomic technologies have given us a lot of opportunities to learn about biological drug targets, microbial-mediated infections are still a problem for global health. The widespread use of antimicrobials and the emergence of resistant genes are the primary causes of this. Another reason is that the pharmaceutical industries are only receiving derivatives of the original drugs because they are rushing to develop additional drugs [9,10].

In recent decades, gene therapy has advanced cancer and congenital disease treatment by optimizing various vector types and introducing novel methods, such as genome editing tools. Because of its high efficiency, low price, and ease of use, the CRISPR/Cas9 system is regarded as one of the most effective tools for genetic engineering. It is anticipated that CRISPR technologies will continue to advance. These methods offer the possibility of treating a wide range of human diseases, despite the fact that there are still a lot of difficult challenges to overcome before they can be safely used in clinical settings.

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