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CRISPR in Clinical and Translational Science: Much Ado about Safety

Abijo T*

Department of Anatomy, College of Medicine, Howard University, Washington DC, USA

Editorial

Over the past few years, there has been an explosion in the development of new technologies that have changed the ways scientists and researchers study diseases. Apart from changing our perspectives about the etiologies and the pathologies of several diseases, these technologies have provided new potential pathways for the treatments of these diseases, most of which are genetic-related, congenital and incurable. Prominent examples are the Next Generation Sequencing (NGS) and the Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR) technologies. Using NGS, scientists can now identify minute variations or polymorphism in the genome of individuals suffering from cancer or other genetic-related diseases, thereby paving ways for personalized medicine. Similarly, CRISPR can be used for specific or random editing of genes to identify new functions of genes and to correct diseased-causing and life-threatening mutations. The fundamental overlapping factor of many of these technologies are the genome and the genes, which underscores the importance of genetics and genomics in several diseases' etiologies and pathologies, including cancer, aging, Alzheimer, Parkinson and other neurodegenerative diseases. While much success has been recorded with the use of these technologies in basic sciences, there has been a slow progression in terms of applications to translational and clinical sciences, a condition which may not be unconnected to safety issues, government regulations and patent challenges. CRISPR technology has gained a lot of recognition based on its potential to treat genetic and congenital diseases. Efforts are now been made to translate the successes recorded with this technology in basic sciences to the clinical sciences. So far, 10 CRISPR-based clinical trials have been approved in China [1], most of which are intended to treat different types of cancer-related diseases and complications arising from the Human Immuno-deficiency Virus (HIV) infections. In Europe, the first approved CRISPR-editing applications will be used to treat patients with beta thalassemia, an inherited blood disorder that affects the body's production of hemoglobin, later in the year [2]. Like Europe, the first CRISPR-editing based treatment has been approved in the United States and researchers at the University of Pennsylvania have begun recruiting patients for the first phase of the trial [1]. The trial, scheduled to start in May, 2018, is a Phase 1 Trial of Autologous T Cells Engineered to Express NY-ESO-1 TCR and CRISPR Gene Edited to Eliminate Endogenous TCR and PD-1 (NYCE T Cells). Although this is a phase 1 clinical trial designed to investigate the safety of this procedure, however when completed and if successful, it will provide a basis and justification for the testing of CRISPR-mediated treatment in people suffering from Multiple Myeloma, Melanoma, Synovial Sarcoma and Myxoid/Round Cell Liposarcoma. Although current trend shows a slow rate of progression in the United States and Europe, It is very important to realize that time is fast running out and many doctors are exhausted using drugs that are just marginally effective. Just like with many cancer drugs, the potential benefit of these technologies outweighs the risks prima facie, especially with increased incidence of cancer and genetic diseases worldwide. It is very important to note that although safety is very important, but in the eyes of a dying man, it may be inconsequential. It is, therefore, time to unleash the power and potentials of these technologies, especially CRISPR as a viable treatment option for a wide-range genetic disease.

References

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***Corresponding author:** Abijo T, Department of Anatomy, College of Medicine, Howard University, Washington DC, USA, Tel: 202-806-6100; E-mail: tomilowo.abijo@bison.howard.edu

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