

Novel Gene Editing Tool Offers Hope for Heart Diseases



Article 1

ABBIE is a cutting-edge technology designed to insert a piece of DNA into a specific site in the genome of a cell. The inserted DNA can be designed to contain one or more genes that can change the function or behavior of the cell. ABBIE can be used to edit the genomic DNA of cells such as stem cells outside of the body (*ex vivo*) and then use the edited cells for therapies to treat various heart diseases such as ischemic heart disease or valvular disorders. In this blog post, we will explain how ABBIE works and how it can be applied to cardiac treatments.

ABBIE stands for “A Binding Based Integrase Enzyme.” It is a synthetic enzyme that is comprised of two main parts: a DNA-binding domain and a DNA-integration domain. The DNA-binding domain recognizes and binds to a specific sequence of DNA in the genome called the target site. The integration domain then efficiently introduces synthesized, exogenous DNA of interest to a given location within a host genome. The donor DNA can be customized to contain any gene or combination of genes. The investigator will leverage the ABBIE targeting system and guide payloads to sites of interest to change the state of the cell.

ABBIE can be used to edit the genome of stem cells, which are cells that have the ability to differentiate into various types of cells in the body. By using ABBIE, researchers can insert genes that can enhance the properties or functions of a desired cell type. Current targets of investigation are pathways that affect specialized cell functions, survival, proliferation, migration, or differentiation. For example, ABBIE can be used to insert genes that can protect stem cells from oxidative stress, inflammation, or apoptosis and thereby increase therapeutic potential. In another example, ABBIE edited cells can restore function of dysfunctional organ tissue that was affected by a genetic disorder.

One of the main applications of ABBIE in cardiology is to use edited stem cells for cell therapies to repair a damaged heart. Cell therapies involve transplanting stem cells or their derivatives into the heart to regenerate or replace the damaged tissue and restore its function. ABBIE can be used to edit stem cells *ex vivo* followed by delivery into the heart of a patient with cardiovascular disease, such as ischemic heart disease or heart failure. ABBIE can insert genes that can improve the engraftment, integration, or differentiation of stem cells into cardiac tissues. For example, ABBIE engineered cells designed to increase the expression of cardiac-specific markers, such as troponin T or connexin-43 facilitate differentiation into cardiomyocytes (heart muscle cells) or form functional connections with the existing cardiac tissue.

ABBIE is a novel and powerful technology designed to edit the genome of cells with high precision and efficiency. This cutting edge technology will contribute to the field of cardiology by enabling new ways of modifying and improving stem cells for cell therapies. ABBIE is currently in early stages of research and development, but it has already shown promising results in animal and human cells in vitro. ABBIE will soon become a reality and make a difference in the lives of millions of people who suffer from heart disease. Stay tuned for more updates on ABBIE and its applications in cardiology!

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