Next Generation Gene Editing Technology

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Synthetic biology / therapeutics

Problem

Current gene editing techniques like CRISPR/Cas face challenges with precision. These methods can inadvertently alter unintended regions of the genome, leading to potential harmful mutations. Additionally, their reliance on creating double-stranded DNA breaks to insert genetic material often triggers DNA repair pathways that can introduce errors. Moreover, there's a practical limitation on the size of genetic sequences that can be introduced, which restricts the application scope, particularly for therapies requiring the insertion of large genes or multiple genetic elements.

Solution

This technology offers a new approach to gene editing, deriving from the capabilities of bacterial IS*1111* and IS*110* family insertion sequences. It combines a specialised transposase enzyme with a custom-designed guide RNA (seekRNA) arising from a defined non-coding region. By its nature, this method is highly precise, substantially reducing the risk of off-target mutations which are common in other gene-editing methods.

A major point of differentiation is this technology's ability to handle the insertion of large DNA sequences, a feat not possible with many current editing tools. This makes it particularly valuable in therapeutic contexts, such as genetic disorders where a corrective gene or a set of genes need to be inserted without triggering the cell's error-prone repair mechanisms.

By avoiding the double-stranded DNA breaks typically required for gene insertion, this technology circumvents the problematic error-prone repair while still ensuring the stable integration of genetic material. This system represents a significant step forward, offering enhanced capabilities for carrying out complex genetic modifications with unprecedented levels of control and reliability.

Intellectual Property Status

This technology is the subject of an Australian provisional patent application.

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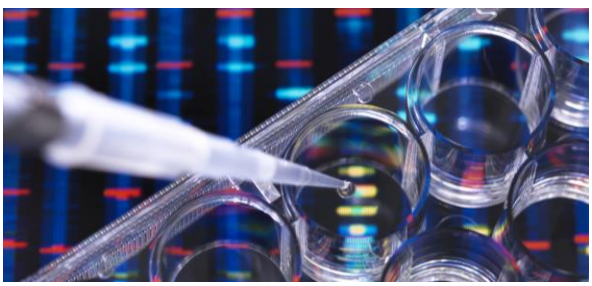
Key Advantages

- The size of the proteins and seekRNA allows them to be delivered in a wide range of forms overcoming the difficulty of CRISPR related technologies.
- This tool is a standalone gene editing insertion without relying in the endogenous cell machinery that allows precise insertion and DNA manipulation.



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