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Increased Packaging Capacity of Viral Vectors for Genome Editing

Stanford researchers have developed a method to overcome the packaging capacity limitation of adeno-associated virus (AAV) Vector CRISPR/Cas9 systems to help treat genetic diseases for which the cargo is larger than 4.7kb.

CRISPR/Cas9 systems have gained momentum in the past decade as a fast, cheap and relatively targeted approach to genome editing. Adeno-associated viral vectors are commonly used to deliver CRISPR/Cas9 due to their efficiency, safety, lower immunogenicity and non-integrative features (i.e. AAV does not integrate into the host genome reducing the likelihood of Cas9 off-target effects). Despite these benefits, one of AAV's shortcomings is its relatively low packaging capacity of 4.7 kb which makes AAV's difficult to use for larger/bulkier cargo.

To overcome this packaging limitation, Stanford researchers developed a delivery construct that increases the packaging capacity of recombinant adeno-associated virus vectors. The improvement creates new therapeutic delivery avenues for genetic diseases that have previously fallen outside of the packaging limitation of AAV's such as hemophilia A, cystic fibrosis, duchenne muscular dystrophy, retinitis pigmentosa, Stargardt Disease, Usher Syndrome 1B, Miyoshi myopathy and more. The advancement also creates opportunities for current CRISPR-based technologies to incorporate the method to improve existing delivery mechanisms.

Stage of Development
In vitro data

Applications

- Genetic editing of larger genes for gene therapy
- Cell engineering in research & industrial applications (i.e. generating cell lines that require the integration of large DNA sequences)

Advantages

- Increased AAV packaging efficiency
- Works in diverse cell types such as T-cells, iPSCs, HSPCs, HBECs etc.
- Can be easily implemented/integrated into current CRISPR/Cas9 systems

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