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CRISPR-Cas13d Therapeutic Platform for Targeted Uveal Melanoma Treatment

Stanford researchers have developed a novel RNA-targeting therapeutic platform using CRISPR-Cas13d to selectively degrade oncogenic mRNA associated with uveal melanoma (UM), an aggressive and treatment-resistant form of eye cancer.

UM is the most common primary intraocular malignancy in adults, With a metastasis rate of nearly 50%, for which there is no effective cure, characterized by limited treatment options and a persistently low 5-year survival rate. Current therapies primarily involve surgical intervention and chemotherapy, which often fail to effectively target the underlying molecular mechanisms driving tumor progression, highlighting the urgent need for tumor-specific therapies.

The CRISPR-Cas13d platform developed at Stanford addresses this gap by using custom-designed guide RNAs (crRNAs) to direct the Cas13d nuclease to degrade cancer-specific mRNA transcripts, leading to significant cytotoxic effects that enhance tumor cell death. This system induces both targeted knockdown and localized collateral RNA cleavage within the tumor cell, resulting in over 97% tumor cell death while minimizing off-target effects in healthy tissue.

This innovative, non-DNA-modifying RNA therapy introduces a highly specific approach to treating uveal melanoma and establishes a modular, scalable platform for expanding Cas13d-based therapeutics across a range of solid tumors. This positions the technology as a potential pipeline-in-a-product, capable of rapidly generating new RNA-targeted therapies for cancers currently considered undruggable.

Stage of Development
Research - in vitro

Applications

- Targeted treatment for uveal melanoma using RNA-guided mRNA degradation
- Modular CRISPR-Cas13d platform for treating other solid tumors
- Research tool for selective mRNA knockdown in undruggable cancers

Advantages

- First-in-class Cas13d therapeutic targeting RNA, not DNA
- High specificity with a >100-fold preference for target vs. non-target transcripts, minimizing off-target effects in healthy tissue
- Non-integrating, transient RNA therapy avoids permanent genetic changes
- Clinically validated LNP delivery enables efficient co-delivery of Cas13d and guide RNAs
- Modular platform adaptable to other cancer targets

Innovators

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