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Oligonucleotide-based therapeutic for targeting Myc-driven cancers

Stanford researchers have discovered that Neat1, a long non-coding RNA, regulates degradation of the MYC protein, revealing a new target for treating MYC-dependent cancers.

Previous studies have demonstrated that elevated MYC expression promotes malignant cellular transformation, uncontrolled proliferation and angiogenesis, thereby enabling oncogenic growth. The extensive occurrence of MYC deregulation across cancer types makes it an attractive therapeutic target with broad clinical potential. Despite this, inhibition of MYC has been challenging due to the absence of a classical druggable binding pocket and its predominantly nuclear localization.

To address this limitation, recent work from the lab of Dr. Mark Kay has uncovered a regulatory pathway that modulates Myc protein degradation. They demonstrated that increased levels of Neat1 RNA, or a defined a short motif of the Neat1 RNA (500 nucleotide sequence) facilitates the interaction between E3 ubiquitin ligase and MYC, resulting in rapid degradation of the MYC protein. Furthermore, introducing antisense oligonucleotides comprising of at least 20 nucleotides complementary to NEAT1 RNA results in a decrease of MYC protein levels. Overall, this approach may expand treatment options to a broad spectrum of MYC-driven tumors that remain largely unresponsive to existing therapies.

Stage of Development
Proof of concept: *in-vitro*

Applications

- Targeted oligonucleotide therapy for MYC-driven cancers
- Research Kits for:
    - increasing or decreasing in MYC protein levels in a cell or organism

    - increasing or decreasing proliferation of a target cell

Advantages

- First-in-class therapy: targets an "undruggable" protein using a unique mechanism of action
- Customizable delivery: supports a wide range of delivery methods, including viral and non-viral approaches
- Combination therapy potential: can be paired with chemotherapies or immunotherapies for enhanced treatment efficacy

Innovators

- Mark Kay
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