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A Novel Mitochondrial-Targeted Treatment for GBM

Stanford researchers have developed Miro1 Reducer, a small molecule that targets the mitochondrial protein Miro1 to treat Glioblastoma, addressing the root cause of tumor growth and immune resistance, and demonstrating significant efficacy in reducing tumor size and improving survival in preclinical models.

Glioblastoma (GBM) is the most common and aggressive primary brain tumor, with an alarming annual incidence of about 16,000 cases and poor survival rate. Current treatment modalities, including surgery, radiation, chemotherapy, and even cutting-edge immunotherapies, have failed to provide substantial improvements in patient outcomes. Despite the therapeutic promise shown by immunotherapies in rodent models, their translation to human patients has faced significant hurdles. Moreover, existing treatments often target the immune cells themselves but fail to address the core mechanisms driving GBM proliferation and resistance. There is an urgent need for improved or alternative treatments that target the root cause of GBM.

To address this need, Stanford researchers have developed Miro1 Reducer, a small molecule targeting a mitochondrial protein to treat GBM. Mitochondria are crucial for cellular homeostasis and the tumor microenvironment, supporting tumor growth and evasion. Cancerous and adjacent immune cells exhibit metabolic changes including the transfer of mitochondria from non-cancerous to cancer cells, facilitating tumor proliferation. Miro1 Reducer addresses this by targeting the mitochondrial protein Miro1, which is key in these processes. By reducing Miro1 expression in GBM cells, this small molecule hinders metabolic adaptations and immune evasion strategies. In a pilot study, Miro1 Reducer has shown remarkable efficacy in the rescue of tumor size and improving survival in GBM mouse models potentially by suppressing mitochondrial DNA-dependent immune activation, preventing mitochondrial transfer, and promoting mitophagy. In summary, Miro1 Reducer offers a novel mitochondrial-targeted therapy for GBM, effectively reducing tumor size and improving survival in

GBM mouse models.

Stage of Development
In vivo. Next steps include SAS and lead optimization, and FDA-enabling studies.

Applications

- Oral drug that Glioblastoma patients can take to prevent tumor growth and improve prognosis
- Targeted therapeutics and personalized treatment for Glioblastoma
- Potential for treating Parkinson's disease and Friedreich Ataxia

Advantages

- Currently, no effective treatments exist on the market for Glioblastoma
- Miro1 Reducer addresses the root cause of immune resistance and tumor proliferation by targeting the mitochondria, unlike existing methods, which primarily target immune cells.

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