

OncoTherapy Science, Inc.

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Publication of a paper describing the anti-cancer effect of a MELK inhibitor OTS167
on neuroblastoma

An article that described the oncogenic properties of MELK in neuroblastoma and ability of OTS167 to suppress the growth of neuroblastoma *in vivo* has been published in *Molecular Cancer Therapeutics*, by a research group of Prof. Cohn and our collaborator Prof. Nakamura's group (currently, director of Cancer Precision Medicine Center, Japanese Foundation for Cancer Research) in the University of Chicago.

Neuroblastoma is a tumor of the sympathetic nervous system, and the second most common solid tumor in childhood. More than half of children with high-risk neuroblastoma do not survive long term despite intensive multimodal treatment, emphasizing the urgent need for more effective and less toxic treatments.

This study demonstrated significant associations between high levels of MELK expression in neuroblastoma tumors with high-risk disease and poor survival. Treatment with the MELK inhibitor OTS167 suppressed proliferation of neuroblastoma cells at low nanomolar concentrations. In neuroblastoma xenograft models, OTS167 suppressed the growth of tumor and prolonged the survival of animals in a minimal residual disease neuroblastoma preclinical model. Moreover, it was revealed that the MELK inhibition increased the sensitivity of neuroblastoma cells to chemotherapy and radiation. Collectively, these findings indicate that MELK is a therapeutic target in neuroblastoma and provide the rationale for further testing of OTS167 as a new treatment option in neuroblastoma.

The paper has been published online in *Molecular Cancer Therapeutics*.

(<http://mct.aacrjournals.org/content/early/2019/01/23/1535-7163.MCT-18-0819>)