

OncoTherapy Science, Inc.

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Important biological roles of a protein kinase TOPK (T-LAK cell-originated protein kinase), for which we are developing small molecule inhibitors, were reported by collaboration between Prof. Yusuke Nakamura's group in The University of Chicago and our research development group in OncoTherapy Science (OTS). This kinase is known to be transactivated in various types of human cancer and play a critical role in the growth of cancer cells. OTS has been developing novel small molecule TOPK inhibitors including OTS964. In this study, the collaborative group discovered that TOPK inhibitors are very effective to the FLT3-ITD-mutated acute myeloid leukemia (AML), which is known to reveal very poor prognosis among AMLs. A TOPK inhibitor almost completely diminished the FLT3 proteins from AML cells including BLAST cells obtained from AML patients. Hence, TOPK inhibitors are considered to be very promising agents to treat FLT3-ITD-mutated AML patients.

Our OTS group participated in this study and has demonstrated that the TOPK inhibitor is efficacious in mouse xenograft model for the FLT3-ITD mutated AML. We will accelerate the development of drugs targeting TOPK to contribute to the improvement of cancer treatment.

The paper was published online in the journal *Oncotarget* on October 2, 2015. (<http://www.impactjournals.com/oncotarget/index.php?journal=oncotarget&page=article&op=view&path%5b%5d=5418&author-preview=46i> )