

OncoTherapy Science, Inc.

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The commencement of Phase I/II clinical study of OTS167,
MELK specific inhibitor, for Acute Myeloid Leukemia patients in USA

OncoTherapy Science, Inc. (President & CEO: Masaharu Mori; hereinafter, "OncoTherapy") announces that we will conduct Phase I/II clinical study of OTS167, a MELK specific inhibitor, for Acute Myeloid Leukemia (AML) patients following the approval of the investigational new drug (IND) application by the US Food and Drug Administration (FDA).

This study is designed to assess the safety and the recommended dose of OTS167 intravenous administration in patients with AML. Once the assessment will be completed, a serial study for the confirmation of POC (Proof of Concept: the clinical verification of the mechanism of action of OTS167) will be performed for various types of leukemia with a high risk of poor prognosis in addition to AML.

This study is planned to be conducted at the University of Chicago and the patient enrollments will commence after the clinical protocol is approved by the Institutional Review Board (IRB) at the University of Chicago Medicine.

The safety, efficacy and pharmacokinetic profiles of intravenous administration of OTS167 in solid cancer patients have been evaluated in the ongoing clinical study at University of Chicago. Additionally, the clinical study to evaluate the bioavailability of OTS167 by oral administration in healthy adults has also been on-going in Australia.

OTS167 is a small-molecule targeted compound that has a potent inhibitory activity against MELK (maternal embryonic leucine zipper kinase), a protein kinase identified as a cancer-specific molecule by genome-wide expression profile analysis. MELK is considered to play important roles in formation and maintenance of cancer stem cells and was shown to be highly expressed in various types of cancers. OTS167 is expected to show the efficacy against cancer stem cells. Experiments using xenograft mice models have already demonstrated potent anti-tumor activities of OTS167 against lung, prostate, breast and pancreatic cancers.

OncoTherapy will proceed with the clinical study for the confirmation of efficacy with careful safety monitoring.