OncoTherapy Science, Inc.
JULY 2, 2013

Announcement of FDA's acceptance of Investigational New Drug (IND) application for Phase I clinical study of OTS167, a novel small molecule compound targeting cancer stem cells

OncoTherapy Science, Inc. (President & CEO: Takuya Tsunoda; hereinafter, "OncoTherapy") announces that the US Food and Drug Administration (FDA) has accepted the investigational new drug (IND) application to initiate Phase I clinical trial of OTS167.

OTS167 is a novel molecular-targeted compound, which has a potent inhibitory activity on MELK (maternal embryonic leucine zeipper kinase), a novel protein kinase identified as a cancer-specific molecule by the genome-wide expression profile analysis.

MELK is not expressed in the important vital organs but highly up-regulated in various types of cancers. Recently, MELK was reported to play the important roles in formation and maintenance of cancer-stem cells in addition to the involvement in tumor growth. Experiments using xenograft mice models demonstrated that OTS167 had potent anti-tumor activities against lung, prostate, breast and pancreatic cancers.

OTS167 is expected to reveal the anti-tumor effect through acting on cancer stem cells of various human cancers.

This clinical trial of OTS167 is a first-in-human Phase I clinical study, designed to assess the safety and pharmacokinetic profiles in patients with advanced solid tumors which failed in standard therapies. The clinical study is planned to be conducted at the University of Chicago and the patient enrollments will be started after the clinical protocol is approved by the Institutional Review Board (IRB) of the University of Chicago.