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ASIA EDITION

Japan grants ODD to Merck's investigational therapy Tepotinib

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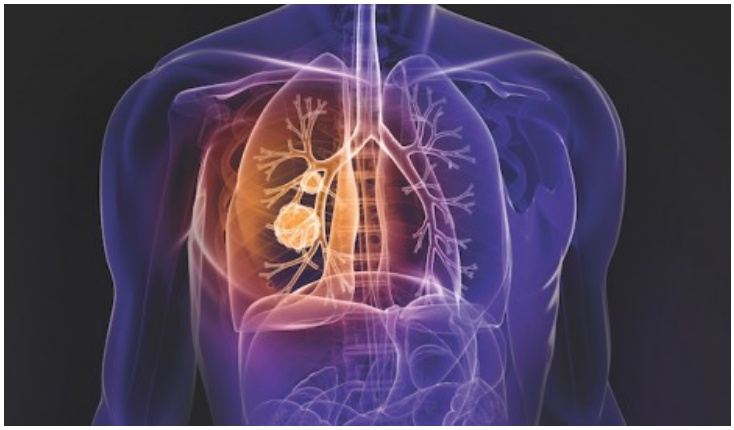
Japanese Ministry of Health, Labour and Welfare grants orphan drug designation (ODD) for diseases that affect fewer than 50,000 patients in Japan, and for which significant unmet medical need exists



Merck, a leading science and technology company, has announced that the Japanese Ministry of Health, Labour and Welfare (MHLW) has granted orphan drug designation (ODD) for its investigational therapy tepotinib for patients with non-small cell lung cancer (NSCLC) harboring *MET* gene alterations.

"Advanced NSCLC harboring *MET* gene alterations is associated with aggressive tumor behavior and poor clinical prognosis," said Luciano Rossetti, Global Head of Research & Development for the Biopharma business of Merck. "This orphan drug designation helps to advance this program within Japan and, coupled with the SAKIGAKE 'fast-track' designation received last year, provides important mechanisms, such as priority review, to quickly deliver this medicine to Japanese patients with this difficult-to-treat disease."

Discovered in-house at Merck, tepotinib is an investigational oral MET inhibitor that is designed to inhibit the oncogenic MET receptor signaling caused by *MET* (gene) alterations, including both *MET* exon 14 skipping alterations and *MET* amplifications, or MET protein overexpression. It has been designed to have a highly selective mechanism of action, with the potential to improve outcomes in aggressive tumors that have a poor prognosis and harbor these specific alterations.

Tepotinib is being investigated in the ongoing VISION study (NCT02864992), which showed preliminary efficacy in patients harboring *MET*ex14 skipping alterations detected by liquid biopsy (LBx) or tissue biopsy (TBx) across different lines of treatment.

Results from the interim analysis of the VISION study were presented in an oral presentation at the 2019 American Society of Clinical Oncology (ASCO) Annual Meeting and the 2019 Japan Society of Medical Oncology (JSMO) Annual Meeting. The use of both LBx and TBx to identify patients for the VISION study is intended to support improved patient selection and is consistent with the company's focus on patient-centric drug development.

Tepotinib is also being investigated in the INSIGHT 2 study (NCT03940703) in combination with the tyrosine kinase inhibitor (TKI) osimertinib in epidermal growth factor receptor (EGFR) mutated, *MET* amplified, locally advanced or metastatic NSCLC having acquired resistance to prior EGFR TKI.

In March 2018, the Japan MHLW granted SAKIGAKE 'fast-track' designation for tepotinib in advanced (stage IIIB/IV) NSCLC harboring *MET*ex14 skipping alterations and, in September 2019, the US Food and Drug Administration (FDA) granted Breakthrough Therapy Designation (BTD) for tepotinib in patients with metastatic NSCLC harboring*MET*ex14 skipping alterations who progressed following platinum-based cancer therapy.