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News Release

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Merck KGaA, Darmstadt, Germany, Receives Fast-Track Designation for Tepotinib in Non-Small Cell Lung Cancer in Japan

- **Japanese Ministry of Health, Labour and Welfare Grants SAKIGAKE designation for tepotinib, Merck KGaA, Darmstadt, Germany's investigational highly selective c-Met receptor tyrosine kinase inhibitor**
- **First regulatory designation for tepotinib**
- **SAKIGAKE designation encompasses the possibility for a target review period of 6 months**

Darmstadt, Germany, March 27, 2018 – Merck KGaA, Darmstadt, Germany, a leading science and technology company, today announced that the Japanese Ministry of Health, Labour and Welfare (MHLW) has granted SAKIGAKE 'fast-track' designation for its investigational molecule tepotinib* for patients with advanced non-small cell lung cancer (NSCLC) harboring MET exon 14 skipping mutations. SAKIGAKE designation promotes research and development in Japan, aiming at early practical application for innovative pharmaceutical products, medical devices, and regenerative medicines, and can reduce a drug's review period down from 12 months to a target of 6 months. This is the first regulatory designation granted to tepotinib.

"This fast-track designation in Japan recognizes the progress we are making with tepotinib as part of our strategic focus on delivering innovative precision medicines to oncology patients," said Luciano Rossetti, M.D., Global Head of Research & Development at the Biopharma business of Merck KGaA, Darmstadt, Germany. "Tepotinib is a highly selective small molecule that – if supported by the outcome of

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further studies – may have the potential to provide particular benefit to NSCLC patients with this genetic mutation."

The SAKIGAKE Designation System is a core component of the MHLW's "Strategy of SAKIGAKE". The system's objective is to designate drugs that have the potential of prominent effectiveness against serious and life-threatening diseases in order to make them available to patients in Japan ahead of the rest of the world.

Tepotinib, discovered in-house at Merck KGaA, Darmstadt, Germany, is an investigational inhibitor of the c-Met receptor tyrosine kinase. The designation consultation on the clinical development program for tepotinib includes a Phase II study exploring the potential of this small molecule in patients with advanced NSCLC harboring MET exon 14 skipping mutations – a population that currently has no approved therapies available. Data from this study will be presented at an upcoming medical congress.

Merck KGaA, Darmstadt, Germany's oncology and immuno-oncology pipeline includes high-quality, selective small molecules, antibodies and antibody-drug conjugates with strong translational research data supporting each drug's clinical development. In addition to tepotinib, the oncology pipeline includes M7824, an investigational bifunctional immunotherapy, designed to simultaneously block two immunoinhibitory pathways (programmed death ligand-1 and transforming growth factor-β), as well as a number of anti-DNA Damage Response molecules (including inhibitors of DNA-PK, ATR and ATM).

**Tepotinib is the recommended International Nonproprietary Name (INN) for the c-Met kinase inhibitor (MSC 2156119J). Tepotinib is currently under clinical investigation and not approved for any use anywhere in the world.*

About Non-Small Cell Lung Cancer (NSCLC)
Lung cancer (both small cell and non-small cell) has been the most common cancer in the world for several decades.[1] Globally, lung cancer is responsible for approximately 1 in every 5 deaths from cancer.[1] The five-year survival rate for people diagnosed with lung cancer that has spread (metastasized) to other areas of the body is 1%.[2] NSCLC is the most common type of lung cancer, accounting for 80 to 85% of all lung cancers.[2] MET Exon 14 skipping mutations occur in 2–3% of lung cancers.[3]

About Tepotinib

Tepotinib is an investigational, highly selective small-molecule inhibitor of the c-Met receptor tyrosine kinase. Alterations of the c-Met signaling pathway are found in various cancer types and correlate with aggressive tumor behavior and poor clinical prognosis. Tepotinib is being investigated in a Phase II study in non-small cell lung cancer.

About Merck KGaA, Darmstadt, Germany

Merck KGaA, Darmstadt, Germany, is a leading science and technology company in healthcare, life science and performance materials. Around 50,000 employees work to further develop technologies that improve and enhance life – from biopharmaceutical therapies to treat cancer or multiple sclerosis, cutting-edge systems for scientific research and production, to liquid crystals for smartphones and LCD televisions. In 2017, Merck KGaA, Darmstadt, Germany, generated sales of € 15.3 billion in 66 countries.

Founded in 1668, Merck KGaA, Darmstadt, Germany, is the world's oldest pharmaceutical and chemical company. The founding family remains the majority owner of the publicly listed corporate group. Merck KGaA, Darmstadt, Germany, holds the global rights to the „Merck" name and brand. The only exceptions are the United States and Canada, where the company operates as EMD Serono, MilliporeSigma and EMD Performance Materials.

References

1. International Agency for Research on Cancer (IARC). GLOBOCAN 2012: Estimated Cancer Incidence, Mortality and Prevalence Worldwide in 2012; Lung Cancer. Available from: http://globocan.iarc.fr/Pages/fact_sheets_cancer.aspx. Last accessed: March 2018.
2. Cancer.net. Lung cancer – non-small cell: statistics. Available from: http://www.cancer.net/cancer-types/lung-cancer-non-small-cell/statistics. Last accessed: March 2018.
3. Reungwetwattana T, Liang Y, et al. The race to target MET exon 14 skipping alterations in non-small cell lung cancer: The Why, the How, the Who, the Unknown, and the Inevitable. *Lung Cancer.* 2017;103:27–37.