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Press Release

U.S. Food and Drug Administration Accepts for Priority Review Deciphera's New Drug Application for Vimseltinib for the Treatment of Patients with Tenosynovial Giant Cell Tumor (TGCT)

Application based on results from the pivotal Phase 3 MOTION study in which vimseltinib demonstrated statistically significant and clinically meaningful objective response rate in this patient population compared to placebo

The U.S. FDA has assigned a target action date of February 17, 2025

EMA has accepted Deciphera's a Marketing Authorization Application for vimseltinib

Osaka, Japan and Waltham, Massachusetts, August 16, 2024 – Ono Pharmaceutical, Co., Ltd. (Headquarters: Osaka, Japan; President: Toichi Takino; "Ono") today announced that the U.S. Food and Drug Administration (FDA) accepted a priority review for the New Drug Application (NDA) on August 14 US time for vimseltinib, a colony stimulating factor 1 receptor (CSF1R), for the treatment of patients with tenosynovial giant cell tumor (TGCT), which is under development by Deciphera Pharmaceuticals, Inc. ("Deciphera"), a wholly-owned subsidiary of Ono. The FDA assigned a Prescription Drug User Fee Act (PDUFA) goal date of February 17, 2025. In mid-July, the European Medicines Agency (EMA) accepted a Marketing Authorization Application (MAA) of vimseltinib and has begun the start of the EMA's centralized review process.

"Building upon positive results from the MOTION pivotal Phase 3 study and following our recent announcement that EMA review of the vimseltinib MAA has begun, we are excited to initiate the regulatory review process in the US and we look forward to working with the FDA to deliver a new treatment option to patients with TGCT" said Steve Hoerter, President and Chief Executive Officer of Deciphera Pharmaceuticals.

The submission is supported by the data from the pivotal Phase 3 MOTION study, evaluating the efficacy and safety of vimseltinib in patients with TGCT not amenable to surgery with no prior anti-CSF1/CSF1R therapy (prior therapy with imatinib or nilotinib allowed), compared to placebo. In the study, vimseltinib demonstrated a statistically significant and clinically meaningful objective response rate (ORR) at Week 25 in the intent-to-treat (ITT) population, as assessed by Blinded Independent Radiologic Review (BIRR) per Response Evaluation Criteria in Solid Tumors version 1.1 (RECIST v1.1), versus placebo (40% in vimseltinib arm vs 0% in placebo arm, $p < 0.0001$). Additionally, vimseltinib demonstrated statistically significant and clinically meaningful improvements versus placebo in all key secondary endpoints. The safety profile of vimseltinib is manageable and safety data from MOTION are consistent with data previously disclosed in the Phase 1/2 clinical trial of vimseltinib*. Results from the MOTION study were presented at the 2024 American Society of Clinical Oncology (ASCO) Annual Meeting and published concurrently in Lancet.

*: Gelberblom, et at. 2024 ASCO Annual Meeting

About MOTION Study

The MOTION study is a two-part, randomized, double-blind, placebo-controlled Phase 3 clinical study to assess the efficacy and safety of vimseltinib in patients with TGCT not amenable to surgery with no prior anti-CSF1/CSF1R therapy (prior therapy with imatinib or nilotinib allowed). The primary endpoint of the study is an objective response rate (ORR) at Week 25 in the intent-to-treat (ITT) population, as assessed by Blinded Independent Radiologic Review (BIRR) per using Response Evaluation Criteria in Solid Tumors version 1.1 (RECIST v1.1), versus placebo. The secondary endpoint includes ORR per tumor volume score (TVS), active range of motion (ROM), physical function, stiffness, quality of life, and pain, all assessed at Week 25.

This study consists of two Parts. In Part 1, patients were randomized to receive either vimseltinib or placebo for 24 weeks. In Part 2, patients randomized to placebo in Part 1 have the option to receive vimseltinib, and all patients receive vimseltinib for a long-term period in an open-label setting.

About Tenosynovial Giant Cell Tumor (TGCT)

TGCT is a rare disease caused by a translocation in colony-stimulating factor 1 (CSF1) gene resulting in overexpression of CSF1 and recruitment of colony-stimulating factor 1 receptor (CSF1R)-positive inflammatory cells into the lesion. TGCT is a rare, non-malignant tumor that develops inside or near joints. TGCT is caused by dysregulation of the CSF1 gene leading to overproduction of CSF1. TGCT is also known as giant cell tumor of the tendon sheath (GCT-TS) or pigmented villonodular synovitis (PVNS), a diffuse-type of TGCT. Although benign, these tumors can grow and cause damage to surrounding tissues and structures inducing pain, swelling, and limitation of movement of the joint. Surgery is the main treatment option; however, these tumors tend to recur, particularly in diffuse-type TGCT. If untreated or if the tumor continually recurs, damage and degeneration may occur in the affected joint and surrounding tissues, which may cause significant disability. For a subset of patients who are not amenable to surgery, systemic treatment options are limited and a new therapeutic option for TGCT is needed.

About Vimseltinib

Vimseltinib is an investigational, oral switch-control tyrosine kinase inhibitor specifically designed to selectively and potently inhibit CSF1R. Vimseltinib has been developed using Deciphera's proprietary switch-control kinase inhibitor platform.

About Deciphera Pharmaceuticals Inc.

As of June 11, 2024, Deciphera became a member of Ono Pharmaceutical Co., Ltd.)

Deciphera is a biopharmaceutical company focused on discovering, developing, and commercializing important new medicines to improve the lives of people with cancer. We are leveraging our proprietary switch-control kinase inhibitor platform and deep expertise in kinase biology to develop a broad portfolio of innovative medicines. In addition to advancing multiple product candidates from our platform in clinical studies, QINLOCK® is Deciphera's switch-control inhibitor approved for the treatment of fourth-line gastrointestinal stromal tumor (GIST). QINLOCK is approved in many countries including the European Union and the United States. For more information, visit www.deciphera.com and follow us on LinkedIn and Twitter (@Deciphera).

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