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Press Release

Ono Announces U.S. Food and Drug Administration Acceptance for Filing of New Drug Application for Tirabrutinib in Patients with Relapsed or Refractory PCNSL

- **Regulatory submission is based on positive results from the Phase 2 PROSPECT Study**
- **FDA sets PDUFA Date of December 18, 2026**

Osaka, Japan and Waltham, Massachusetts, February 16, 2026 – Ono Pharmaceuticals Co., Ltd. (Headquarters: Osaka, Japan; President and COO: Toichi Takino; "Ono"), today announced that the U.S. Food and Drug Administration (FDA) has accepted for filing the New Drug Application (NDA) under the accelerated approval pathway for tirabrutinib, a highly selective irreversible, second generation Bruton's tyrosine kinase inhibitor (BTK), for the treatment of relapsed or refractory primary central nervous system lymphoma (R/R PCNSL). The FDA has set an action date of December 18, 2026, under the Prescription Drug User Fee Act (PDUFA).

"R/R PCNSL is a rare and aggressive form of non-Hodgkin lymphoma with particularly poor clinical outcomes. Patients often experience difficulty and delay in diagnosis, and once they are diagnosed, there is a high unmet need for a treatment with a favorable safety profile," said Matthew L. Sherman, M.D., Chief Medical Officer of Deciphera. "The FDA's acceptance of tirabrutinib's NDA for filing is an exciting milestone as it brings us one step closer to our goal of providing patients with R/R PCNSL an important new treatment option."

"We are very pleased that the NDA for tirabrutinib has been accepted for filing," said Toichi Takino, President and COO of Ono. "This is an important milestone on the way to expanding our commercial pipeline and achieving our goal of becoming a global specialty pharma. Tirabrutinib's potential to address unmet patient needs embodies our corporate philosophy and we will continue to focus on developing and delivering innovative medicines to benefit patients worldwide."

The NDA is supported by the positive results from the Phase 2 PROSPECT study, presented at the 2025 American Society for Clinical Oncology (ASCO) Annual Meeting, in which tirabrutinib demonstrated an overall response rate of 67%, a complete response rate of 44%, and a manageable safety profile. If approved, tirabrutinib will be the first BTK inhibitor therapy commercially available in the U.S. for the treatment of patients with R/R PCNSL, and the third commercial therapy for Ono group available in the U.S. The study is currently enrolling patients with R/R PCNSL in a global Phase 3 randomized trial, which will serve as a confirmatory study for this indication. (ClinicalTrials.gov NCT07104032.)

About Tirabrutinib

Tirabrutinib, discovered and developed by Ono Pharmaceutical Co., Ltd., is a highly potent selective BTK inhibitor. Signaling through the B-cell receptor (BCR) regulates cellular proliferation and activation, and promotes survival, differentiation, and clonal expansion of B-cells. The BCR signaling pathway plays an important role in a number of B-cell malignancies.

In Japan, tirabrutinib was approved in March 2020 for the treatment of R/R PCNSL and launched under the tradename of VELEXBRU® in May 2020. It was subsequently approved for the treatment of Waldenstrom macroglobulinemia and lymphoplasmacytic lymphoma in August 2020. Tirabrutinib was approved for the treatment of R/R PCNSL in South Korea in November 2021 and in Taiwan in February 2022.

About PCNSL

PCNSL is a rare and aggressive extra-nodal non-Hodgkin lymphoma (NHL) that is confined to the brain parenchyma, spinal cord, eye, or leptomeninges without systemic involvement. The annual incidence rate of PCNSL is approximately five cases per 1,000,000 people in the U.S. The rate can further increase among immuno-compromised people aged 65 years and older. The signs and symptoms presented in patients with PCNSL vary depending on the neuroanatomical site of the lesion, and include cranial neuropathy, neuropsychiatric symptoms, symptoms associated with increased intracranial pressure, seizures, ocular symptoms, headache, dysmotility, cranial neuropathy, and radiculopathy. There is a high unmet need for treatment with a favorable safety profile, and data guiding therapeutic approaches are very limited. Despite recent progress resulting in the improvement of clinical outcomes in newly diagnosed patients with PCNSL after an induction treatment, approximately 20 to 30 percent of patients are refractory to the initial treatment, and up to 60 percent of patients will eventually relapse. To learn more about R/R PCNSL, please visit navigatingpcnsl.com.

About Deciphera Pharmaceuticals Inc.

Deciphera, a member of Ono Pharmaceutical Co., Ltd., is a biopharmaceutical company focused on discovering, developing, and commercializing important new medicines to improve the lives of people with cancer. Deciphera is leveraging its 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 Deciphera's platform in clinical studies, QINLOCK® (ripretinib) is Deciphera's switch-control kinase inhibitor approved in many countries including the European Union and the United States for the treatment of adult patients with advanced gastrointestinal stromal tumor (GIST) who have received prior treatment with 3 or more kinase inhibitors, including imatinib. ROMVIMZA® (vimseltinib) is a kinase inhibitor approved in the United States for adult patients with symptomatic tenosynovial giant cell tumor (TGCT) for which surgical resection will potentially cause worsening functional limitation or severe morbidity, and in the European Union for adult patients with TGCT associated with clinically relevant physical function deterioration and in whom surgical options have been exhausted or would induce unacceptable morbidity or disability. For more information, visit www.deciphera.com and follow us on LinkedIn and X (@Deciphera).

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