FDA Approves Oncopeptides´ PEPAXTO® (melphalan flufenamide) for Patients with Triple-Class Refractory Multiple Myeloma

—U.S. Commercial Launch Underway—

WALTHAM — **March 1, 2021** — Oncopeptides AB (publ) (Nasdaq Stockholm: ONCO), a global biotech company focused on the development of therapies for difficult-to-treat hematological diseases, today announced that the U.S. Food and Drug Administration (FDA) has approved PEPAXTO® (melphalan flufenamide), known during clinical development as melflufen, in combination with dexamethasone, for the treatment of adult patients with relapsed or refractory multiple myeloma who have received at least four prior lines of therapy and whose disease is refractory to at least one proteasome inhibitor, one immunomodulatory agent, and one CD38-directed monoclonal antibody. This indication has been granted under accelerated approval based upon the HORIZON trial. PEPAXTO is the first anticancer peptide-drug conjugate approved in multiple myeloma.

"While the treatment landscape for multiple myeloma has dramatically improved in recent years, once patients become resistant to existing classes of therapy they can face a very guarded prognosis," said Paul G. Richardson, MD, Clinical Program Leader and Director of Clinical Research at the Jerome Lipper Multiple Myeloma Center, Dana-Farber Cancer Institute. "Research has shown melphalan flufenamide to be a novel and innovative therapeutic option, which is active in refractory disease and has manageable toxicity, with the convenience of being administered by infusion once a month. Based on our findings, melphalan flufenamide is an important addition to the treatment armamentarium, with the potential to meaningfully improve outcomes in an area of important unmet medical need."

The HORIZON study, evaluating intravenous PEPAXTO in combination with dexamethasone, included heavily pre-treated patients with a poor prognosis. This multi-center single arm study evaluated 157 patients with relapsed or refractory multiple myeloma, of whom 97 were triple-class refractory and had received at least four prior lines of treatment. The Overall Response Rate for the patients within this group of patients with refractory multiple myeloma was 23.7 percent and the Median Duration of Response was 4.2 months. Of the subset of 97 patients, 41% had EMD (n=40), an aggressive and resistant disease associated with poor prognosis.

"We are proud to bring forward the first anticancer peptide-drug conjugate approved by the FDA for multiple myeloma," said Jakob Lindberg, Chief Scientific Officer at Oncopeptides. "PEPAXTO uses innovative technology that links a peptide carrier to a cytotoxic agent. The conjugated agent is a highly lipophilic compound, which allows it to be rapidly distributed into cells. The compound then leverages amino peptidases that are overexpressed in Multiple Myeloma cells, causing the release of the cytotoxic payload."

Marty Duvall, Chief Executive Officer at Oncopeptides added, "The accelerated U.S. approval of PEPAXTO is a key step forward in fulfilling Oncopeptides' core mission, to bring hope to patients in their

battle against difficult-to-treat hematological diseases. Moving ahead, our focus is to further advance PEPAXTO. We look forward to receiving top line data from the phase 3 OCEAN study in the second quarter. This comparative study is designed to support a future supplementary New Drug Application to expand the label."

PEPAXTO was approved under accelerated approval, which allows for earlier approval of drugs that treat serious conditions, and that fill an unmet medical need based on a surrogate endpoint. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

Oncopeptides expects PEPAXTO to become commercially available in the U.S. within approximately two weeks.

About the HORIZON Study

In total, 157 multiple myeloma patients have been enrolled in the pivotal phase 2 HORIZON study, evaluating intravenous PEPAXTO in combination with dexamethasone. The approval was based on a subgroup of HORIZON patients (n=97) who had received at least four prior lines of therapy and whose disease is refractory to at least one proteasome inhibitor, one immunomodulatory agent, and one CD38-directed monoclonal antibody. In this subset of Triple Class Refractory patients, the Overall Response Rate (ORR) was 23.7 percent and Median Duration of Response (DOR) was 4.2 months.

The most common adverse reactions (≥20%; Grade 1-4) were fatigue (55%), nausea (32%), diarrhea (27%), pyrexia (24%), and respiratory tract infection (24%). The most common laboratory abnormalities (Grade 1-4) were leukocytes decrease (99%), platelets decrease (99%), lymphocytes decrease (97%), neutrophils decrease (95%), hemoglobin decrease (84%), and creatinine increase (68%).

About Multiple Myeloma

Multiple myeloma is a cancer of plasma cells, a type of white blood cell which produces antibodies to help fight infection. Multiple myeloma causes cancer cells to accumulate in the bone marrow. Approximately 7 per 100,000 Americans per year are diagnosed with multiple myeloma, making it a rare disease. A growing subset of this population is becoming triple-class refractory (TCR). The number of patients diagnosed with multiple myeloma is growing and the number of cases diagnosed annually is expected to almost double in 20 years. The average age for diagnosis is 70 years of age, and there is currently no cure.

About PEPAXTO®

PEPAXTO® (melphalan flufenamide) is the first anticancer peptide-drug conjugate for patients with

triple-class refractory Multiple Myeloma who have received at least four prior lines of therapy. PEPAXTO uses innovative technology that links a peptide carrier to a cytotoxic agent, resulting in a lipophilic compound. Due to its lipophilicity, PEPAXTO is distributed into cells. PEPAXTO is designed to leverage aminopeptidases, which are overexpressed in multiple myeloma cells and cause the release of the cytotoxic agents. PEPAXTO is administered as a once monthly thirty-minute infusion.

INDICATION

PEPAXTO is indicated in combination with dexamethasone for the treatment of adult patients with relapsed or refractory multiple myeloma who have received at least four prior lines of therapy and whose disease is refractory to at least one proteasome inhibitor, one immunomodulatory agent, and one CD38-directed monoclonal antibody. This indication is approved under accelerated approval based on response rate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

Limitation of Use

PEPAXTO is not indicated and is not recommended for use as a conditioning regimen for transplant outside of controlled clinical trials.

IMPORTANT SAFETY INFORMATION

PEPAXTO is contraindicated in patients with a history of serious allergic reaction to melphalan flufenamide or melphalan.

PEPAXTO may cause thrombocytopenia, which may lead to hemorrhage. Monitor platelets at baseline, during treatment, and as clinically indicated. Monitor more frequently during the first 2 months of treatment with PEPAXTO. Do not administer PEPAXTO if the platelet count is less than 50 x 10^9/L. Withhold PEPAXTO until platelet count is 50 x 10^9/L or greater and resume at same or reduced dose based on duration of interruption. Adjust dose and/or dose schedule based on signs and symptoms of bleeding.

PEPAXTO may cause neutropenia, which may lead to infection. Monitor neutrophil counts at baseline, during treatment, and as clinically indicated. Monitor more frequently during the first 2 months of treatment with PEPAXTO. Do not administer PEPAXTO if absolute neutrophil count is less than 1 x 10^9/L. Withhold PEPAXTO until absolute neutrophil count is 1 x 10^9/L or greater and resume at same or reduced dose based on duration of interruption. Consider leukocyte growth factor as clinically appropriate.

PEPAXTO may cause anemia. Monitor red blood cell counts at baseline, during treatment, and as clinically indicated. Monitor more frequently during the first 2 months of treatment with PEPAXTO. Treat anemia as clinically indicated. Dosage modification and dose delay of PEPAXTO may be required to allow for recovery of red blood cells.

Patients taking PEPAXTO experienced infections, including fatal infections. Consider antimicrobials as clinically appropriate.

Nonclinical safety studies with melphalan flufenamide at dosages exceeding the recommended dose for PEPAXTO were associated with mortality. The safety and efficacy of PEPAXTO has not been established for use as a conditioning regimen in patients receiving transplant.

Secondary malignancies such as myelodysplastic syndromes or acute leukemia have been reported in patients with multiple myeloma who were treated with PEPAXTO. Monitor patients long term for the development of secondary malignancies.

Based on its mechanism of action, PEPAXTO can cause fetal harm when administered to a pregnant woman because it is genotoxic and targets actively dividing cells. Advise pregnant women of the potential risk to a fetus. Advise females of reproductive potential to use effective contraception during treatment with PEPAXTO and for 6 months after the last dose. Advise males with female partners of reproductive potential to use effective contraception during treatment with PEPAXTO and for 3 months after the last dose.

The most common adverse reactions (≥20%; Grade 1-4) were fatigue (55%), nausea (32%), diarrhea (27%), pyrexia (24%), and respiratory tract infection (24%).

Special Considerations

Use in Pregnancy

Based on the mechanism of action, PEPAXTO can cause fetal harm when administered to a pregnant woman. There are no available data on PEPAXTO use in pregnant women to evaluate for a drug-associated risk. PEPAXTO is a genotoxic drug. Advise a woman of childbearing potential of the potential risks to the fetus.

Lactation

There is no information regarding the presence of melphalan flufenamide or its metabolites in human breast milk, or the effects on the breastfed child or on milk production. Because of the potential for

serious adverse reactions in a breastfed child, advise women not to breastfeed during treatment with PEPAXTO and for 1 week after the last dose.

Females and Males of Reproductive Potential

PEPAXTO can cause fetal harm when administered to a pregnant woman. Advise females of reproductive potential to use effective contraception during treatment with PEPAXTO and for 6 months after the last dose.

PEPAXTO may damage the developing male germ cells, resulting in possible genetic fetal abnormalities. Advise males with a female partner of reproductive potential to use effective contraception during treatment with PEPAXTO and for 3 months after the last dose.

PEPAXTO can cause amenorrhea in premenopausal women and result in infertility. Based on findings of melphalan flufenamide in animals, PEPAXTO may impair male fertility. Alkylating drugs, such as PEPAXTO, can also cause irreversible testicular suppression in patients.

Please find the full prescribing information available for viewing here:
https://pepaxto.com/docs/pepaxto_pi.pdf

About Oncopeptides

Oncopeptides is a global biotech company committed to developing targeted therapies for patients facing hard-to-treat hematological diseases. Oncopeptides has one U.S. FDA approved product, PEPAXTO® (melphalan flufenamide), known during clinical development as melflufen. PEPAXTO is approved for patients with triple-class refractory multiple myeloma and was evaluated in several clinical studies including the pivotal Phase 2 HORIZON study and is currently being evaluated in the confirmatory Phase 3 OCEAN study. Oncopeptides' headquarters is in Stockholm, Sweden, with a U.S. headquarters in Boston, Massachusetts. In addition to Boston, Oncopeptides has a footprint in Los Altos, California, another U.S. biotech hub. For more information, please visit our corporate website at https://oncopeptides.se/en/. You may also visit our U.S. website at https://www.oncopeptides-us.com/en and follow us on our U.S. social media channels, Twitter and LinkedIn.

PEPAXTO® is a trademark of Oncopeptides AB (publ).

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