

FDA approves Oncopeptides' PEPAXTO® (melphalan flufenamide) for patients with relapsed or refractory multiple myeloma

STOCKHOLM — February 26, 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, also known 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.

Oncopeptides will begin promoting PEPAXTO to healthcare professionals across the U.S. immediately and expects a labeled product in distribution centers and specialty pharmacies within approximately two weeks. PEPAXTO is the first anticancer peptide-drug conjugate approved by the FDA. The product has been granted accelerated approval based on the phase 2 HORIZON study in relapsed or refractory multiple myeloma.

“The accelerated approval of PEPAXTO in the US is an important milestone for Oncopeptides, and a major step ahead in fulfilling our mission, to bring hope to patients with difficult-to-treat hematological diseases, through innovative science”, says Marty J Duvall, Chief Executive Officer at Oncopeptides AB. “Moving ahead, our focus is to further advance PEPAXTO. We look forward to receiving top line data from the phase 3 OCEAN-study in relapsed refractory multiple myeloma, in the second quarter. The comparative study with pomalidomide, is designed to support a future supplementary New Drug Application to expand the label”.

“When we listed Oncopeptides on Nasdaq Stockholm, we promised to establish melflufen as an attractive treatment option for patients with multi-resistant disease. With the approval of PEPAXTO, that has finally become reality”, said Jakob Lindberg, Chief Scientific Officer and former CEO at Oncopeptides. “I am immensely proud of the relentless dedication of our organization and development partners around the world who have made this journey possible”.

“Melphalan flufenamide is a novel and innovative therapeutic option which is active in patients with multiple myeloma who have a refractory disease, and the product has a manageable toxicity”, says Professor Ola Landgren, Chief of Myeloma Program and Leader of Experimental Therapeutics Program, Division of Hematology, Sylvester Comprehensive Cancer Center, University of Miami Health System in Miami, Florida. “Melphalan flufenamide will complement existing treatment regimens and contribute to address the growing unmet medical need among patients with relapsed or refractory multiple myeloma”.

The HORIZON study evaluating intravenous melflufen 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% and the Median Duration of Response was 4.2 months. Furthermore, melflufen in combination with dexamethasone demonstrated activity in a subset of patients with Extra Medullary Disease (41%), an aggressive and resistant disease associated with a poor prognosis.

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The information in the press release is information that Oncopeptides is obliged to make public pursuant to the EU Market Abuse Regulation. The information was submitted for publication, through the agency of the contact person above, on February 26, 2021, at 23:30 (CET).

Webcast for investors, analysts and the media

The Company will host a webcast on March 1, 2021, at 07:30 (CET), including presentations by CEO Marty J Duvall, CSO Jakob Lindberg, CMO Klaas Bakker, and CFO Anders Martin-Löf.

The conference call will be streamed through the following web link;

https://tv.streamfabriken.com/pressconference_oncopeptides_2021

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About Multiple Myeloma

Multiple myeloma is a cancer that impacts 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 are each year diagnosed with multiple myeloma, making it a rare disease. A growing subset of this population is becoming triple-class refractory. This means that their disease is refractory to at least one proteasome inhibitor, one immunomodulatory agent, and one CD38-directed monoclonal antibody. 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, and there is currently no cure.

About the HORIZON Study

In total, 157 multiple myeloma patients have been enrolled in the pivotal phase 2 HORIZON study, evaluating intravenous melflufen in combination with dexamethasone. The approval of PEPAXTO was based on a subgroup of HORIZON patients (n=97) that were refractory to at least one treatment in each of the three standard-of-care classes: proteasome inhibitor, immunomodulatory agent, and CD38-directed monoclonal antibody and had received at least four prior lines of treatment. In this subset of patients, the Overall Response Rate (ORR) was 23.7% and Median Duration of Response (DOR) was 4.2 months. The most frequent adverse reactions (≥10%; 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 PEPAXTO®

PEPAXTO® (melphalan flufenamide, also known as melflufen) is the first anticancer peptide-drug conjugate for patients with relapsed or refractory multiple myeloma. 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 cytotoxic agents. PEPAXTO is administered as a once monthly thirty-minute infusion.

In the US PEPAXTO is indicated in combination with dexamethasone for the treatment of adult patients with triple class 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-monoclonal directed antibody. PEPAXTO® is a registered trademark in the U.S.

About Oncopeptides

Oncopeptides is a global biotech company focused on the development of targeted therapies for difficult-to-treat hematological diseases. The Company has recently been granted accelerated approval by the U.S. FDA for PEPAXTO (melphalan flufenamide, also known as melflufen), in relapsed or refractory multiple myeloma. PEPAXTO is the first drug originated from the Company's proprietary PDC-platform and is evaluated in a comprehensive clinical study program, including the ongoing phase 3 OCEAN study. PEPAXTO is the first anticancer peptide-drug conjugate for patients with relapsed or refractory multiple myeloma. The product uses innovative technology that links a peptide carrier to a cytotoxic agent, resulting in a lipophilic compound. Due to its lipophilicity, it 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. Oncopeptides' global Headquarters is in Stockholm, Sweden and the U.S. Headquarters is situated in Boston, Mass. The company is listed in the Mid Cap segment on Nasdaq Stockholm with the ticker ONCO. More information is available on www.oncopeptides.com.