

FDA grants Priority Review of melflufen for patients with triple-class refractory multiple myeloma

STOCKHOLM — August 29, 2020 — Oncopeptides AB (publ) (Nasdaq Stockholm: ONCO) today announces that the US Food and Drug Administration, FDA, has granted priority review for Oncopeptides' New Drug Application seeking approval of melflufen (INN melphalan flufenamide), in combination with dexamethasone for the treatment of adult patients with multiple myeloma whose disease is refractory to at least one proteasome inhibitor, one immunomodulatory agent and one anti-CD-38 monoclonal antibody, (i.e., triple-class refractory multiple myeloma patients). The FDA has set a PDUFA-date (Prescription Drug User Fee Act), which is the target date for their review of the New Drug Application, to February 28, 2021.

The submission is based on the results from the pivotal phase 2 study HORIZON, evaluating intravenous melflufen in combination with dexamethasone in patients with relapsed refractory multiple myeloma, (RRMM).

“This is very exciting news. It is an important milestone for Oncopeptides, and a major step in making melflufen available for patients with multiple myeloma, who desperately need new treatment options”, says Marty J Duvall, CEO of Oncopeptides AB. “I am looking forward to a continuing dialogue with the FDA while we make the product available to RRMM patients in the US through an expanded access program, in an FDA approved trial called sEAPort.”

A Priority Review designation means that FDA's goal is to take action on an application within 6 months (compared to 10 months under standard review). They will direct overall resources to the evaluation of applications for drugs that, “if approved, would be significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications”.

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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 August 29, 2020 at 10.00 (CET).

About melflufen

Melflufen (INN melphalan flufenamide) is a first in class peptide-drug conjugate (PDC) that targets aminopeptidases and rapidly releases alkylating agents into tumor cells. Melflufen is rapidly taken up by myeloma cells due to its high lipophilicity and is immediately hydrolyzed by peptidases to release an entrapped hydrophilic alkylator payload. Aminopeptidases are overexpressed in tumor cells and are even more pronounced in advanced cancers and tumors with a high mutational burden. In vitro, melflufen is 50-fold more potent in myeloma cells

than the alkylator payload itself due to the increased intracellular alkylator concentration. Melflufen displays cytotoxic activity against myeloma cell lines resistant to other treatments, including alkylators, and has also demonstrated inhibition of DNA repair induction and angiogenesis in preclinical studies. In the pivotal phase 2 HORIZON study melflufen plus dexamethasone demonstrated encouraging efficacy and a clinically manageable safety profile in heavily pretreated patients with relapsed refractory multiple myeloma, with primarily hematologic Adverse Events (AE) and a low incidence of non-hematologic AEs.

About Oncopeptides

Oncopeptides is a pharmaceutical company focused on the development of targeted therapies for difficult-to-treat hematological diseases. The company is focusing on the development of the lead product candidate melflufen, a first in class peptide-drug conjugate (PDC) that targets aminopeptidases and rapidly releases alkylating agents into tumor cells. Melflufen (INN melphalan flufenamide) is in development as a new treatment for the hematological malignancy multiple myeloma and is currently being tested in multiple clinical studies including the pivotal phase 2 HORIZON study and the ongoing phase 3 OCEAN study. Based on the results from the HORIZON study Oncopeptides has submitted a New Drug Application (NDA) to the U.S. Food and Drug Administration, FDA, for accelerated approval of melflufen in combination with dexamethasone for treatment of adult patients with triple-class refractory multiple myeloma. 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.