

Oncopeptides initiates U.S. Expanded Access Program with melflufen in triple-class refractory multiple myeloma

STOCKHOLM — October 1, 2020 — Oncopeptides AB (publ) (Nasdaq Stockholm: ONCO) announced today that the open-label Expanded Access Program, sEAPort, for eligible U.S. patients, is formally open. Melflufen (INN melphalan flufenamide), is currently being evaluated in several clinical studies as a treatment for patients with triple-class refractory multiple myeloma. The sEAPort program is available to adults, age 18 and older, who have received at least two prior lines of therapy and whose multiple myeloma is refractory to at least one proteasome inhibitor, one immunomodulatory drug and one anti-CD38 monoclonal antibody, (i.e., triple-class refractory multiple myeloma patients).

The Expanded Access Program was initiated following the Company's June 30 submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration, FDA, for accelerated approval of melflufen in combination with dexamethasone for the treatment of adult patients with triple-class refractory multiple myeloma. The NDA and the sEAPort program are primarily supported by data from the pivotal phase 2 HORIZON study, which demonstrates that melflufen in combination with dexamethasone, has a potential to provide a therapeutic option for patients with relapsed refractory multiple myeloma who are hard to treat and have a poor prognosis, including patients with triple-class refractory multiple myeloma and patients with extramedullary disease.

"Despite therapeutic advances, multiple myeloma remains incurable," said Paula O'Connor, U.S. Head of Medical Affairs at Oncopeptides. "There is an urgent need for more therapies as patients become multi-resistant earlier in their treatment journey. Our Expanded Access Program enables us to provide access to melflufen as a potential treatment for eligible patients while our application is under review by the U.S. Food and Drug Administration."

EAPs are designed to provide patients living with serious or life-threatening conditions access to investigational medicines when no comparable or satisfactory treatment options are available, alternative therapies have been exhausted or the patient is ineligible for ongoing interventional trials.

Forty to fifty medical sites in the U.S. are expected to enroll 100-200 patients in the sEAPort program.

More information about our clinical trials can be found at www.clinicaltrials.gov

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This information was submitted for publication at 08.00 (CET), October 1, 2020.

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.