

## Full data set of Oncopeptides phase 2 HORIZON study in multiple myeloma published in the Journal of Clinical Oncology

**STOCKHOLM — December 9, 2020** — Oncopeptides AB (publ) (Nasdaq Stockholm: ONCO), a pharmaceutical company focused on the development of targeted therapies for difficult-to-treat hematological diseases, today announces that the pivotal phase 2 HORIZON study evaluating intravenous melflufen (INN melphalan flufenamide) in combination with dexamethasone in relapsed refractory multiple myeloma, has been published in the peer-reviewed Journal of Clinical Oncology.

The phase 2 HORIZON data are the basis for the ongoing priority review of the New Drug Application to the US Food and Drug Administration FDA, for accelerated approval of melflufen in combination with dexamethasone in triple-class refractory multiple myeloma patients, who are refractory to at least one proteasome inhibitor, one immunomodulatory drug and one anti-CD38 monoclonal antibody.

“The results from the HORIZON study demonstrate that melflufen in combination with dexamethasone, has a potential to provide a therapeutic option for patients who are difficult to treat and have a poor prognosis, including patients with triple class refractory myeloma and patients with extramedullary disease”, says Klaas Bakker, MD, PhD, Chief Medical Officer, Oncopeptides AB. “These patients have limited, or no treatment options left. The introduction of a new treatment class may represent a potentially important alternative”.

The phase 2 HORIZON study is a pivotal, single-arm, multicenter, phase 2 study evaluating the safety and efficacy of melflufen in combination with dexamethasone in patients with relapsed refractory multiple myeloma. The study included 157 heavily pretreated patients, who had received >2 earlier lines of therapy with immunomodulatory drugs and proteasome inhibitors and were refractory to pomalidomide and/or daratumumab. The HORIZON study population includes subgroups of patients who were triple-class refractory and/or had extramedullary disease and/or had cytogenetic high-risk features.

### Summary of results:

<b>30</b>	<b>Intention to Treat (n=157)</b>	<b>Triple Class Refractory (n=119)</b>	<b>Extra Medullary Disease (n=55)</b>
Overall Response Rate (ORR)	29%	26%	24%
Median Progression Free Survival (PFS))	4.2 months	3.9 months	2.9 months
Median Overall Survival (OS)	11.6 months	11.2 months	6.5 months
Responding patients	n=45	n=31	n=13
Median Duration of Response (DOR)	5.5 months	4.4 months	5.5 months
Median Progression Free Survival (PFS)	8.5 months	8.5 months	17.3 months

The publication is available on; <https://ascopubs.org/doi/full/10.1200/JCO.20.02259>

**For more information, please contact:**

Klaas Bakker, MD, PhD, Chief Medical Officer, Oncopeptides

Mail: [klaas.bakker@oncopeptides.com](mailto:klaas.bakker@oncopeptides.com)

Mobile: +44 7818 523903

Rein Piir, Head of Investor Relations, Oncopeptides

E-mail: [rein.piiir@oncopeptides.com](mailto:rein.piiir@oncopeptides.com)

Cell phone: +46 70 853 72 92

The information in this press release was submitted for publication on December 9, 2020 at 22:00 (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 lead product candidate melflufen, is a first in class peptide-drug conjugate that targets aminopeptidases and rapidly releases alkylating agents into tumor cells. Melflufen is in development as a new treatment for the hematological malignancy multiple myeloma and is 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 a New Drug Application has been submitted 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. The FDA, has granted the New Drug Application a priority review, with a PDUFA date of February 28, 2021. 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](http://www.oncopeptides.com).