

Oncopeptides reports positive results from full data set of Phase 2 HORIZON trial in triple-class refractory multiple myeloma patients

STOCKHOLM — June 15, 2020 — Oncopeptides AB (Nasdaq Stockholm: ONCO) announces today that the full top-line results from the pivotal phase 2 HORIZON study, evaluating intravenous melflufen in combination with dexamethasone in patients with relapsed refractory multiple myeloma (RRMM), have been presented at the ongoing 25th European Hematology Association meeting, EHA. The results support the NDA submission to the US Food and Drug Administration, FDA, for accelerated approval of melflufen. The Company is on track to submit the application to FDA by the end of Q2, 2020.

Oncopeptides will host a webcast on Tuesday, June 16 at 10.00 (CET) to provide comments on the full top-line results. The webcast can be followed via the link: <https://financialhearings.com/event/12977>

Summary of results

End Points	Intention to Treat (n=157)	Triple Class Refractory (n=119)	Extra Medullary Disease (n=55)
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

All data were confirmed by the Independent Review Committee (IRC), with only minimal discordance.

Melflufen is a first-in-class anticancer peptide-drug conjugate that rapidly delivers an alkylating payload into tumor cells. The results from the HORIZON study demonstrates that melflufen in combination with dexamethasone, have the potential to provide a therapeutic option for patients with relapsed refractory multiple myeloma (RRMM) that are hard to treat and have a poor prognosis, including patients with triple class refractory myeloma and patients with Extramedullary Disease. Responses were durable and often deepened with prolonged treatment, suggesting that patients could benefit from staying on treatment for as long as possible. The results are consistent with previously reported data, while no new safety concerns were identified. The full poster presentation is available on Oncopeptides' website: <https://oncopeptides.se/en/eha-2020-poster/>.

“The HORIZON data is an important milestone for Oncopeptides and further validates our Peptide-Drug Conjugate platform”, says Klaas Bakker, MD and CMO of Oncopeptides. “The results are in line with results from previous interim analyses. Notably, the PFS of 8.5 months in responding patients (both all treated and triple-class refractory), was materially higher than the DOR, which is explained by the relatively long-time it took for patients

to respond (median 1.9 months). This is very encouraging for patients with an unmet need; ultimately this is the period patients benefit from treatment with melflufen. The fact that the treatment also seems to be well tolerable makes this a potentially attractive treatment option for a fast-growing patient population with a significant unmet medical need”.

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About the HORIZON (OP-106) study

In the pivotal phase 2 HORIZON study 157 multiple myeloma patients have been enrolled and evaluated. The study was fully recruited in October 2019 and the final data cut was made on January 14th. The patients in the study are refractory to pomalidomide and/or daratumumab after failing on immunomodulatory drugs (IMiDs) and proteasome inhibitors (PIs). The HORIZON study population includes subgroups of patients who were triple-class refractory and/or had EMD and/or had cytogenetic high-risk features.

About melflufen

Melflufen (INN melphalan flufenamide) is a first-in-class anti-cancer peptide-drug conjugate that rapidly delivers an alkylating payload into tumor cells. Melflufen is rapidly taken up by myeloma cells due to its high lipophilicity and is immediately cleaved by peptidases to deliver an entrapped hydrophilic alkylator payload. Peptidases play a key role in protein homeostasis and feature in cellular processes such as cell-cycle progression and programmed cell death. 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.

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 anti-cancer peptide-drug conjugate that rapidly delivers an alkylating payload into tumor cells. Melflufen 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. Oncopeptides' headquarters is in Stockholm, Sweden with its U.S. headquarters 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.