

Oncopeptides submits a New Drug Application to the FDA for accelerated approval of melflufen in triple-class refractory multiple myeloma patients

STOCKHOLM — June 30, 2020 — Oncopeptides AB (publ) (Nasdaq Stockholm: ONCO) today announces that the Company submits a New Drug Application (NDA) to the U.S. Food and Drug Administration, FDA, for accelerated 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-CD38 monoclonal antibody (i.e., triple-class refractory multiple myeloma patients).

Melflufen is the lead candidate coming out of the Oncopeptides' proprietary PDC-platform. The product is a first-in-class aminopeptidase-targeting peptide-drug conjugate that rapidly delivers an alkylating payload into tumor cells. 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).

The results from the HORIZON study demonstrates that melflufen in combination with dexamethasone, has a potential to provide a therapeutic option for patients with RRMM that are hard to treat and have a poor prognosis, including patients with triple class refractory myeloma and patients with extramedullary disease (EMD). The responses in the HORIZON study were durable and often deepened with prolonged treatment, suggesting that patients could benefit from staying on treatment for as long as possible.

“I am very proud and humbled by the organizations ability to timely submit the NDA for accelerated approval of melflufen. This is a major milestone for Oncopeptides and is a result of dedicated research and development activities throughout the last decade”, says Jakob Lindberg CEO of Oncopeptides. “I would like to express my sincere gratitude to all patients, co-workers, investigators and shareholders who have provided relentless support to enable a novel treatment option for a fast-growing patient population with a significant unmet medical need”.

Following the submission to the FDA Oncopeptides will initiate an Expanded Access Program (EAP) in the U.S. to enable melflufen treatment for patients with a significant unmet medical need.

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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 June 30, 2020 at 08.00 (CET).

About the HORIZON study

In total 157 multiple myeloma patients have been enrolled and evaluated in the pivotal phase 2 HORIZON study. The study was fully recruited in October 2019, the final data cut was made on January 14th and the final data was presented at the EHA meeting in June. The patients in the study were 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 extramedullary disease and/or had cytogenetic high-risk features.

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.

About melflufen

Melflufen (INN melphalan flufenamide) is a first-in-class aminopeptidase-targeting 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 hydrolyzed 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.

Expanded access policy

The preparations for an Expanded Access Program (EAP) in the US are well underway and the program will open in Q3. Oncopeptides encourages awareness of and participation in its clinical trials and believes that participating in clinical trials is a good way for patients to access investigational drugs prior to regulatory approval. Individuals interested in participating in clinical trials for melflufen may visit <https://www.oncopeptides.se/en/our-clinical-trials-summary/> for information about ongoing clinical trials. Patients are encouraged to consult their physician regarding the possibility of participating in one of the ongoing clinical trials.

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 aminopeptidase-targeting 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.