

Data from Oncopeptides Melflufen Clinical Development Program Presented at SOHO 2019 Annual Meeting

Stockholm – September 11, 2019 – Oncopeptides AB (Nasdaq Stockholm: ONCO) announced today that clinical trial data were presented at the Society of Hematologic Oncology (SOHO) 2019 Annual Meeting in Houston, TX, USA. In an oral presentation, data from the pivotal Phase 2 HORIZON clinical trial were presented as part of the meeting’s multiple myeloma session. Additionally, two posters focused on the ANCHOR and HORIZON clinical trials evaluating melflufen in RRMM were displayed at the meeting. The data presented are encouraging and show continued momentum for Oncopeptides’ melflufen.

In the pivotal Phase 2 HORIZON trial for which interim results were presented, melflufen demonstrated promising activity in patients with RRMM, many of whom also have extramedullary disease (EMD). In the study, patients demonstrated an overall response rate (ORR) of 28% and a clinical benefit rate (CBR) of 40%. 86% of patients evaluated achieved disease stabilization (SD) or better. Melflufen was generally well tolerated with manageable toxicity. The data have previously been presented at EHA annual meeting in Amsterdam.

Dr Christopher Maisel, MD, comments

“These Interim data from the HORIZON clinical trial demonstrate the potential for melflufen to be a novel therapeutic option for patients with RRMM,” said Christopher Maisel, MD, of Texas Oncology and Baylor Sammons Cancer Center in Dallas, TX. “I am encouraged by these results, and glad to see that there is a selective alkylator with efficacy and manageable toxicity in RRMM patients, as this patient population remains a significant unmet need.”

CEO, Jakob Lindberg comments

“The selection of these data for oral presentation at the esteemed SOHO 2019 Annual Meeting reinforce the scientific rigor of Oncopeptides’ clinical development program supporting our lead candidate melflufen, and we are excited about this first presentation of these interim data to U.S. clinician audiences,” said Jakob Lindberg, CEO of Oncopeptides. “As we look toward our planned NDA submission in the first quarter of 2020, we look forward to providing continuing updates on the HORIZON clinical trial at additional upcoming medical congresses.”

The ongoing Phase 2 HORIZON clinical trial will serve as the basis for Oncopeptides’ planned submission of a New Drug Application (NDA) to the FDA for accelerated approval of melflufen in the treatment of patients with triple-class refractory multiple myeloma. The company is targeting submission of the NDA in the first quarter of 2020.

The full oral presentation and posters presented at the SOHO 2019 Annual Meeting can be found on the company webpage under:

www.oncopeptides.com / Investors & media / Presentations / SOHO 2019

Oral Presentation: HORIZON (OP-106) Study of Melflufen in Patients with Relapsed/Refractory Multiple Myeloma (RRMM) Refractory to Daratumumab and/or Pomalidomide: Updated Efficacy and Safety

Interim data from the pivotal Phase 2 HORIZON clinical trial was featured in an oral presentation by Christopher Maisel, M.D., Baylor Scott & White Charles A. Sammons Cancer Center, Dallas, Texas, USA. The presentation was featured as one of only two oral abstracts selected for the meeting's official session on multiple myeloma. These interim data from the HORIZON trial were presented as an encore presentation of the results following their initial presentation at the European Hematology Association (EHA) 2019 Annual Congress.

Summary of HORIZON Interim Data

Melflufen continues to demonstrate promising activity in patients with RRMM, many of whom also have EMD. According to these interim study results, patients demonstrated an ORR of 28% and a clinical benefit rate (CBR) of 40%.

Median PFS is 4.0 months in the ongoing study and duration of response (DOR) is 4.4 months. The majority of patients evaluated (86%) achieved SD or better.

Treatment was generally well tolerated with manageable toxicity, nonhematologic AEs were infrequent and the rate of discontinuation due to AEs was low. Treatment-related SAEs occurred in 20% of patients and were most commonly febrile neutropenia (5%) and thrombocytopenia (2%).

The median age of patients in the clinical study was 64 years. 62% of patients in the study had high-risk cytogenetics, 29% of patients were ISS stage III and 60% of the patients had EMD. The median number of prior lines of therapy was five and the median time since initial diagnosis was 6.2 years.

All patients in the study were investigator assessed as non-responsive or non-tolerant to immunomodulatory drugs (IMiDs) and proteasome inhibitors (PIs). 36% of patients had received 3+ treatment regimens over the last 12 months. 91% of patients were double class refractory (IMiD + PI) and 79% anti-CD38 mAb refractory. 74% of the patients were triple class (IMiD + PI + Anti-CD38 mAb) refractory and 59% were alkylator refractory. 98% of the patients were refractory in last line of therapy.

About the OP-106 HORIZON Clinical Trial

Patient recruitment in the HORIZON study is ongoing. The interim data presented at SOHO 2019 is based on a data cut-off dated May 6, 2019, with 121 patients treated. 108 patients had received two or more cycles of treatment. The goal is to include 150 patients in the study. The patients in the study are refractory to pomalidomide and/or daratumumab after failing on IMiDs and PIs.

More information can be found at: <https://clinicaltrials.gov/ct2/show/NCT02963493?term=melflufen&rank=2>

Poster Presentation: HORIZON (OP-106) Study of Melflufen in Patients With Relapsed/Refractory Multiple Myeloma (RRMM) Refractory to Daratumumab and/or Pomalidomide: Updated Efficacy and Safety

Efficacy and safety from the ongoing HORIZON clinical trial was also featured as a poster (Poster MM-250) at the SOHO 2019 Annual Meeting, providing an update on the full clinical trial design, patient characteristics and initial conclusions presented in the corresponding oral presentation.

Poster Presentation: ANCHOR (OP-104) Study of Melflufen and Dexamethasone Plus Bortezomib or Daratumumab in Patients With Relapsed/Refractory Multiple Myeloma (RRMM) Refractory to an IMiD and/or a Proteasome Inhibitor (PI): Phase 1 Update

An update on the Phase 1/2 ANCHOR clinical trial evaluating melflufen in combination with either bortezomib or daratumumab for the treatment of RRMM was also selected as a poster presentation (Poster MM-168) at the SOHO 2019 Annual Meeting. According to the data presented, the combination of melflufen with either bortezomib or daratumumab is well tolerated and evolving efficacy is encouraging in both combinations, with 90% of patients still on treatment. The ANCHOR study is ongoing with active recruitment of patients to the 40-mg melflufen dose level.

About the OP-104 ANCHOR study

ANCHOR is a Phase 1/2 trial where melflufen and dexamethasone is dosed in combination with either bortezomib or daratumumab. All patients must have 1-4 prior lines of therapy and be refractory (or intolerant) to an immunomodulatory agent (IMiD) or a proteasome inhibitor (PI) or both. The trial is currently open for enrollment at multiple sites globally.

More information can be found at: <https://clinicaltrials.gov/ct2/show/NCT03481556?term=melflufen&rank=4>

In the bortezomib combination arm (Regimen A) patients cannot be refractory to a PI and in the daratumumab combination arm (Regimen B) patients cannot be previously exposed to any anti-CD38 therapy. Patients will be treated until documented disease progression or unacceptable toxicity. The primary objective of the phase 1 part of the study is to determine the optimal dose of melflufen, up to a maximum of 40 mg, and dexamethasone in combination with bortezomib or daratumumab. Additional patients per regimen are recruited in the phase 2 part of the trial where the primary objective is ORR.

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About Melflufen

Melflufen is a lipophilic peptide-conjugated alkylator that rapidly delivers a highly cytotoxic payload into myeloma cells through peptidase activity. It belongs to the novel class Peptidase Enhanced Cytotoxics (PEnc), which is a family of lipophilic peptides that exhibit increased activity via peptidase cleavage and have the potential to treat many cancers. Peptidases play a key role in protein homeostasis and feature in cellular processes such as cell-cycle progression and programmed cell death. Melflufen is rapidly taken up by myeloma cells due to its high lipophilicity and immediately cleaved by peptidases to deliver an entrapped hydrophilic alkylator payload. In vitro, melflufen is 50-fold more potent in myeloma cells than the alkylator payload itself due to the peptidase cleavage, and induces irreversible DNA damage and apoptosis. 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 cancers. The company is focusing on the development of the lead product candidate melflufen, a novel lipophilic peptide conjugated alkylator, belonging to a new class of drugs called Peptidase Enhanced Cytotoxics (PEnc). Melflufen is in development as a new treatment for the hematological cancer multiple myeloma, including the Phase 2 pivotal trial HORIZON currently underway and a global confirmatory Phase 3 trial (OCEAN) continuing enrollment. Oncopeptides' headquarters is located in Stockholm, Sweden, and the company is listed in the Mid Cap segment on Nasdaq Stockholm with the ticker ONCO.

For more information please visit www.oncopeptides.com.