

New Interim Data in RRMM Patients with Extramedullary Disease from the Pivotal Phase 2 Horizon-study presented at International Myeloma Workshop

Stockholm – September 15, 2019 - Oncopeptides AB (Nasdaq Stockholm: ONCO) announced today updated interim efficacy and safety data from the ongoing pivotal Phase 2 study, HORIZON, at the 17th International Myeloma Workshop (IMW) meeting in Boston, Massachusetts, USA.

The data focused on patients with extramedullary disease (EMD) and was presented in an oral presentation by Professor Paul G. Richardson. Oncopeptides will host a conference call to review this data on Monday, September 16, 2019, at 08.30 (CET).

The results presented at IMW 2019 by Professor Paul G Richardson in the plenary session “Late Breaking Abstracts” under the title: Activity of Melflufen in RRMM Patients with Extramedullary Disease in the Phase 2 Study (OP-106 HORIZON) – Promising Results in a High-Risk Population, are based on a data cut in the HORIZON study dated July 30, 2019 when 136 patients were treated compared to previously reported data including 121 patients. Based on literature, HORIZON represents the largest clinical cohort to date of myeloma patients with extra medullary disease (EMD).

Relapsed-refractory multiple myeloma (RRMM) patients with extramedullary disease (EMD) is a very difficult to treat patient population. In recent single-agent studies (with or without steroids) including RRMM patients with EMD have shown overall response rates (ORR) between 0 - 17%. Only daratumumab has shown any relevant clinical activity with a response rate of 17% in daratumumab naïve myeloma patients with EMD.

In the HORIZON study, an overall response rate of 23% was achieved with treatment with melflufen and dexamethasone in RRMM patients with EMD. These patients were mainly penta refractory (had undergone at least five prior treatments and were resistant to at least one proteasome inhibitor, one IMiD, and anti-CD38 treatment). The EMD patients who responded to the treatment had a median survival (OS) of 18.5 months compared to a median survival of 5.1 months for the EMD patients who did not respond to treatment. The difference observed suggests a clear clinical benefit for these patients.

Professor Paul G Richardson comments

“We are experiencing a significant increase in the number of relapsed and refractory myeloma patients with extramedullary disease and without effective treatment options. The observation in the HORIZON trial, that melflufen shows activity in these patients and may impact favorably on survival outcome, is very encouraging. In my view, given the relative lack of therapeutic alternatives for these patients, moving rapidly into melflufen-based combination strategies in patients with extramedullary disease, as well as exploring earlier lines of therapy, should be the next priority”, said Professor Paul G Richardson, Professor of Medicine at Harvard Medical School and Clinical Program Leader, Director of Clinical Research at the Jerome Lipper Multiple Myeloma Center Dana-Farber Cancer Institute in Boston, Massachusetts, USA.

Overall Conclusions From the Presentation

- As of July 30, 2019, 136 patients had been treated in HORIZON with melflufen and dexamethasone. 44 out of the 136 patients had confirmed EMD diagnosis at baseline
- 91% of EMD patients were triple-class refractory and 73% penta refractory
- Similar ORR in non-EMD and EMD patients with an ORR of 27% and 23% respectively
- Response rates in EMD patients was seemingly higher than historical studies
- Median OS was 5.8 months for patients with EMD and 11.6 months for patients without EMD
- Median OS in EMD responders and non-responders was 18.5 months and 5.1 months, respectively
- Median OS in non-EMD responders and non-responders was 17.2 months and 8.5 months, respectively
- The treatment was generally well tolerated, with manageable toxicity with a low overall incidence of nonhematologic Adverse Events (AE) including infections; no treatment-related deaths

The full presentation from IMW is available on the company's website under:

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

Conference call for investors, analysts and the media

CEO Jakob Lindberg will review of the results presented at IMW, on Monday September 16, 2019 at 08:30 (CET).

The conference call will also be streamed via a link on the company website: www.oncopeptides.com.

Phone numbers for participants from:

Sweden: +46 8 505 583 68

Europe: +44 3333 009 271

USA: +1 833 823 05 87

For further information, please contact:

Jakob Lindberg, CEO of Oncopeptides

E-mail: jakob.lindberg@oncopeptides.com

Telephone: +46 8 615 20 40

Rein Piir, Head of Investor Relations at Oncopeptides

E-mail: rein.piir@oncopeptides.com

Cell phone: +46 70 853 72 92

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 September 15, 2019 at 14.30 (CET).

About the OP-106 HORIZON study

Patient recruitment in the HORIZON study is ongoing. The interim data presented at IMW 2019 is based on a data cut-off dated July 30, 2019, with 136 patients treated. 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 immunomodulatory drugs (IMiDs) and proteasome inhibitors (PIs).

Oncopeptides has initiated preparations for submitting a New Drug Application (NDA) for accelerated approval of melflufen in the US based on the available HORIZON data. The detailed plan for the filing process is still under development, but Oncopeptides currently targets to submit the application to FDA during the first quarter of 2020. This could then lead to the first melflufen market approval in the US in 2020.

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

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 (OP-103 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.