

New Data Presented at 24th EHA Congress from Oncopeptides' Phase 1/2 ANCHOR Trial Evaluating Melflufen in Relapsed/Refractory Multiple Myeloma (RRMM)

Stockholm - 14 June 2019 - Oncopeptides AB (Nasdaq Stockholm: ONCO), announced today new data presented at the 24th Congress of the European Hematology Association (EHA) in Amsterdam, including updated interim data from the ongoing phase 1/2 ANCHOR study and data from its phase 1/2 O-12-M1 study.

On 16 June 2019, interim data from Oncopeptides' ongoing, pivotal Phase 2 HORIZON trial will also be presented as an oral presentation by 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.

The full posters presented at EHA can be found on the company webpage under:

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

Comment from CEO Jakob Lindberg

"It is very encouraging to see consistently high response rates with deepening responses over time for melflufen in combination with proteasome inhibition and anti-CD38 therapies in the ongoing ANCHOR study in patients with relapsed/refractory multiple myeloma. The progression free survival similarly looks encouraging with some patients having been on treatment for more than a year and at this point in time only one patient in each arm has experienced disease progression. Together with the emerging safety and tolerability profile, this strengthens our belief that melflufen could add value for myeloma patients also as part of combination regimens," said Jakob Lindberg, CEO of Oncopeptides. "The results from the O-12-M1 study demonstrate that melflufen can offer disease stabilization and favourable time to the next treatment. It is strategically important from a health economic perspective and supports the potential that melflufen may play an important role in the treatment of patients with RRMM."

Overall Conclusions – ANCHOR Poster Presentation

- Based on interim data from the ANCHOR trial in patients with RRMM, the combination of melflufen and dexamethasone with either bortezomib or daratumumab is well tolerated.
- Evolving efficacy is encouraging in both combinations, with 90% of patients still on treatment.
- For patients that had completed two or more cycles of therapy the overall response rate (ORR) was 100% for the bortezomib combination and 82% for the daratumumab combination. Responses with both combinations improved with continued therapy.
- No dose limiting toxicities (DLTs) have been observed across both regimens and dose levels.
- Grade 3/4 adverse events (AEs) were mostly hematologic and all were clinically manageable.

Comment from Dr. Enrique Ocio

"The updated interim data from ANCHOR that is investigating the combination of melflufen and dexamethasone with either bortezomib or daratumumab in patients with relapsed/refractory multiple myeloma,

show very encouraging efficacy both in terms of response rates and emerging durability of the responses. It will be exciting to continue to follow these trial results as more patients enroll in the study and continue treatment with combination regimens based on melflufen,” said Dr. Enrique Ocio at Hospital Universitario Marques de Valdecilla, Santander, Spain.

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.

Summary of the OP-104 ANCHOR interim data

Melflufen in combination with bortezomib – Regimen A

At the time of data cutoff, 8 May 2019, five patients had been treated with melflufen (three with 30 mg, two with 40 mg). The median age was 73 years, with a median of two prior lines (range, 2-4), and no patient had achieved CR in any previous line. All patients had relapsed / refractory disease and two of the five patients were last-line refractory (disease progression while on therapy).

The median treatment duration was 7.4 months (range, 2-11 months). Four patients were ongoing on treatment while one discontinued treatment due to disease progression after 10 months. Two patients achieved very good partial response (VGPR) and three patients achieved partial responses (PR) for an ORR of 100%.

Melflufen in combination with daratumumab – Regimen B

At the time of data cutoff, 8 May 2019, twenty-four patients had been treated with melflufen (six with 30 mg, eighteen with 40 mg). The median age was 60 years with a median of two prior lines of therapy.

The median treatment duration was 7.9 months (range, 0-11 months) and 1.2 months (range, 0-9 months) on 30 mg and 40 mg, respectively. All six patients on 30 mg and 16 of the 18 patients on 40 mg were still ongoing. Two patients discontinued treatment due to physician’s decision (one due to lack of response).

Subgroup	No. of Patients								Percentage of Patients	
	sCR	CR	VGPR	PR	MR	SD	PD	NA	ORR	CBR
Total (n=24)	0	1	4	4	4 a)	2	0	9	60%	87%
Patients with 2+ completed cycles therapy (n=11)	0	1	4	4	1 b)	1	0	0	82%	91%

CBR, clinical benefit rate; CR, complete response; MR, minimal response; NA, no assessment at time of data cutoff; ORR, overall response rate; PD, progressive disease; PR, partial response; sCR, stringent CR; SD, stable disease; VGPR, very good PR.

a) Includes 3 unconfirmed MR.

b) Includes 1 unconfirmed MR.

Overall Conclusions – O-12-M1 Poster Presentation

- Melflufen plus dexamethasone treatment results in disease stabilization in 76% of RRMM patients, which translates to a median Time To Next Treatment (TTNT) of 7.9 months (10.6 months when censoring at time of death), which compares favorably with other relevant trials.
- A median OS of 20.7 months in an advanced RRMM population, suggesting that melflufen therapy is associated with a long-term benefit and allows patients to receive further treatment to control disease.
- Results support previously presented data showing the promising efficacy profile of melflufen for the treatment of RRMM.

Comment from Dr. Sara Brighen

“The data presented at EHA from the phase 1/2 study, O-12-M1, is very important. A high proportion of patients, 76%, show disease stabilization translating into 7.9 months as median time to next treatment. The median overall survival of 20.7 months observed in this advanced RRMM patient population suggests that melflufen therapy may be associated with a long-term benefit, allowing patients to receive further treatment to control disease significantly, thereby improving patient outcomes and their quality of life,” said Dr. Sara Brighen at the Division of Hematology, University of Torino, Italy.

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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 14, 2019 at 09.40 (CET).

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.
Visit www.oncopeptides.com for more information.