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Interim data from the ongoing HORIZON trial with melflufen presented in an oral presentation at the 60th American Society of Hematology Meeting

Stockholm – December 3, 2018 – Oncopeptides AB (Nasdaq Stockholm: ONCO) announced today that Professor Paul G. Richardson presented updated interim data with melflufen (Ygalo®) from the ongoing HORIZON trial at the 60th ASH meeting in San Diego, California, USA.

Overall conclusions

The updated phase II data with melflufen in late-stage relapsed-refractory multiple myeloma patients refractory to pomalidomide and/or daratumumab after failing on immunomodulatory drugs (IMiDs) and proteasome inhibitors (PIs) shows:

- *An Overall Response Rate (ORR) of 33%, in patients where 46% had received 3+ regimens of treatments over the last 12 months*
- *A vast majority of patients, 84%, achieved disease stabilization (SD or better)*
- *The first evaluation of Progression Free Survival (PFS) shows a median of 4.0 months in the ongoing trial with a median of 6.4 months for responding patients*
- *Activity was observed regardless of underlying refractory status and the traditional prognostic factor albumin was a strong predictor of ORR*
- *The treatment was well tolerated with mainly reversible and manageable hematological adverse events. The incidence of non-hematological toxicity was low*

Professor Paul G. Richardson comments

"Over the last decade, initial treatments in myeloma have been radically improved, and in particular with the advent of the widespread adoption of novel agent combination approaches as well as continuous therapy. However, the number of patients who become resistant to immunomodulatory agents, proteasome inhibitors and anti-CD38 monoclonal antibodies is increasing, and there is thus a clear need for effective treatment options with new mechanisms of action. Melflufen is a first in class peptidase-enhanced compound that does not share resistance pathways seen with currently used treatments and has general manageable toxicity with favorable tolerability. Melflufen is showing considerable promise in the relapsed and refractory setting, with clinical development ongoing" said Paul Richardson MD, the RJ Corman Professor of Medicine at Harvard Medical School and Director of Clinical Research at the Jerome Lipper Multiple Myeloma Center, Dana-Farber Cancer Institute, Boston, USA.

About the HORIZON study

Patient recruitment to the study is ongoing. The interim data presented at the ASH meeting is based on a data cut-off dated October 22ed 2018 with 83 patients treated and 82 patients included in the response analysis. The patients in the study are refractory to pomalidomide and/or daratumumab after failing on IMiDs and PIs.

Summary of the HORIZON interim data

The study continues to develop positively in this heavily pretreated patient group that is refractory to pomalidomide and/or daratumumab after failing on IMiDs and PIs with few remaining treatment options.

- 61% of patients in the study had high-risk cytogenetics, 36% of patients were ISS stage III, the median number of prior lines of therapy was 5 and the median time since initial diagnosis was 6.5 years.
- All patients in the study were investigator assessed as non-responsive or non-tolerant to IMiDs and PIs, 100% of patients were also refractory to pomalidomide or daratumumab, 60% were refractory to IMiDs, PIs and anti-CD38, 55% were alkylator refractory and 93% had disease progression on or within 60 days of completion of the last therapy.
- Analysis of the preliminary efficacy results showed an ORR of 32,9% and that 84,1% of the patients achieved disease stabilization (SD or better).

Overall response rate (N=82)								
	ORR	sCR	VGPR	PR	MR	SD	PD	NE
total, N=82	32,9%	1,2%	11%	20,7%	6,1%	45,1%	14,6%	1,2%

- Subgroup analysis suggests that response does not vary across refractory subsets but rather with the underlying disease and health status of the patient (confirming the observation made in Oncopeptides phase II study O-12-M1).

This study confirms earlier results from the O-12-M1 study in a more resistant patient population. The efficacy results in this interim analysis are encouraging with an ORR of 32,9%.

Melflufen showed a manageable safety and tolerability profile. Treatment-related grade 3/4 AEs were reported in 62 (75%) patients with the majority being hematological. Treatment-related non-hematological grade 3/4 AEs were rare with infections in only 7% of patients. 13% of the patients discontinued treatment due to AEs.

About melflufen

Melflufen (Ygalo®), a peptide conjugated alkylator belonging to a novel class of peptidase-enhanced compounds, targets multiple myeloma (MM) cells with a unique mechanism of action. Aminopeptidases are enzymes found in all cells but are over-expressed in several cancers including MM. Melflufen selectively targets MM cells through aminopeptidase-driven accumulation. In vitro experiments show a 50-fold enrichment of the active substance in MM cells compared with administration of equal amount of an alkylator not enriched by aminopeptidases. The enrichment results in selective cytotoxicity (increased on-target potency and decreased off-target toxicity), and that resistance pathways of existing myeloma treatments (including alkylators) is overcome. Melflufen also demonstrates strong anti-angiogenic properties.

Melflufen in clinical development

Melflufen (Ygalo®) has been used to treat late-stage RRMM patients in both phase I and phase II clinical studies (O-12-M1) with favorable results. Currently, melflufen is being studied in four clinical trials for the treatment of multiple myeloma. The current studies are OCEAN, HORIZON, ANCHOR and BRIDGE.

The current clinical study program is intended to demonstrate better results from treatment with melflufen compared to established alternative drugs for patients with late-stage multiple myeloma. Melflufen could potentially provide physicians with a new treatment option for patients suffering from this serious disease.

Melflufen has been investigated in the treatment of late-stage relapsed refractory multiple myeloma (RRMM) patients. This was done in the clinical study O-12-M1 where strong final results were reported in December 2017. Currently, four clinical studies are ongoing with melflufen.

OCEAN is Oncopeptides´ pivotal Phase III study where melflufen is compared directly with current standard of care, pomalidomide, in late-stage RRMM patients.

HORIZON is a Phase II study that studies the effect of melflufen in late-stage RRMM patients with few or no remaining established treatment options. Updated interim data from this study were presented at ASH in December 2018.

ANCHOR is a phase I/II study where melflufen is administered in combination with either bortezomib or daratumumab in RRMM patients. The results of this study aim to create understanding and knowledge among treating physicians for how melflufen can be used in combination with these drugs. In addition, the results could open up for the use of melflufen in earlier lines of treatment. The first interim data from the study was presented in a poster presentation on December 1, 2018 at ASH.

BRIDGE is a phase II study, where melflufen is used in RRMM patients with impaired renal function. This is a positioning study to show melflufen's treatment profile in these patients.

About Oncopeptides

Oncopeptides is a pharmaceutical company developing drugs for the treatment of cancer. The company is focusing on the development of the lead product candidate melflufen (Ygalo®), a peptide conjugated alkylator, belonging to a new class of drugs called Peptidase Enhanced Compounds. Melflufen is intended as an effective treatment of hematological cancers, and in particular multiple myeloma. The goal with the current clinical study program is to demonstrate better results from treatment with melflufen compared with established alternative drugs for patients with late-stage multiple myeloma. Melflufen will potentially provide physicians with a new treatment option for patients suffering from this serious disease.

Visit **www.oncopeptides.com** for more information.

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