**Stockholm, Sweden** **Press release June 15, 2018**

# Oncopeptides present updated interim data from the ongoing HORIZON trial with Ygalo® at the 23rd Congress of The European Hematology Association (EHA)

**Stockholm – June 15, 2018 - Oncopeptides AB (Nasdaq Stockholm: ONCO) announced today that they are presenting updated interim data with Ygalo® (melflufen) from the ongoing HORIZON trial at the 23rd EHA congress in Stockholm.**

**The updated phase II-data show a clinical data set with an Overall Response Rate (ORR) of 32.1% and a Clinical Benefit Rate (CBR) of 39.3% with Ygalo® in relapsed/refractory multiple myeloma patients refractory to pomalidomide and/or daratumumab after failing on immunomodulatory drugs (IMiDs) and proteasome inhibitors (PIs).**

**The data are presented in a poster that can be found at:** www.oncopeptides.se/en/presentations/eha

## CEO comments

"In HORIZON, we are studying the activity of Ygalo® in myeloma patients that have failed on all, or the majority of, treatments that are currently in use. In addition, half the patients in HORIZON are ISS stage III and half the patients have high-risk cytogenetics. This means that the patients are very ill, since both parameters are strong predictors of poor treatment outcome. To our knowledge this is the highest combined number in any study in myeloma to date. Despite all this, we see a tumor response in 32% of patients, disease stabilization in 84% of patients, positive initial indication of the duration of the treatment effect as well as a manageable safety profile for Ygalo®. We have made the decision to expand the HORIZON trial to further understand the efficacy of Ygalo® in this very difficult to treat patient population", said Jakob Lindberg, CEO of Oncopeptides.

## Professor Paul G. Richardson comments

"With an increasing number of patients with highly resistant myeloma there is a real need for additional treatment options based on new mechanisms of action. Ygalo®, a peptidase-enhanced compound, with its potent activity, manageable tolerability and lack of shared resistance mechanisms with other modalities, is a promising molecule that is making encouraging progress in clinical development" said Professor Paul Richardson, Harvard Medical School at the Dana-Farber Cancer Institute, Boston, USA.

## About the HORIZON study

The study recruitment is ongoing. The interim data presented at the EHA congress are based on a data cut-off dated May 10th 2018 with 62 patients treated. The patients in the study should be refractory to pomalidomide and/or daratumumab after failing on IMiDs and PIs.

## Conclusions regarding HORIZON

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.

- 54% of patients in the study had high-risk cytogenetics, 46% of patients were ISS stage III, the median number of prior lines of therapy was 5.5 and the median time since initial diagnosis was 6.1 years.
- 100% of patients were refractory to pomalidomide or daratumumab, 98% had disease progression on or within 60 days of completion of the last therapy, 89% were double-refractory to IMiD:s and PI:s and 56% were refractory to both pomalidomide and daratumumab.
- Analysis of the preliminary efficacy results showed an ORR of 32.1%, a CBR of 39.3% and that 84% of the patients achieved disease stabilization (SD or better).

| **Overall response rate (N=56)** | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | ORR | CBR | CR | VGPR | PR | MR | SD | PD |
| total, N=56 | 32.1% | 39.3% | 2% | 9% | 21% | 7% | 45% | 16% |

- Subgroup analysis suggests that response does not vary across refractory subsets but rather with the underlying disease and health status of the patient (in line with the observation made in Oncopeptides phase II study O-12-M1).
- Time-to-next-treatment was maintained compared to the previous line of therapy without the deterioration normally seen in myeloma patients.
- In the previous line of therapy, 75% of the patients were treated with antibody-based therapies or 2nd/3rd generation PI:s and IMiD:s, and 46% received triple combination therapies.

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 32,1% and a CBR of 39,3%.

Ygalo® showed a manageable safety and tolerability profile. Treatment-related grade 3/4 AEs were reported in 48 (77%) patients with the majority being hematologic. Treatment-related non-hematologic grade 3/4 AEs were rare with infections in only 6% of patients.

## About Ygalo®

Ygalo® is an alkylating peptide, belonging to the novel class of Peptidase Enhanced Compounds (PEnCs), targeting the multiple myeloma (MM) transformation process with a unique mechanism of action. Aminopeptidases are heavily over-expressed in MM cells and are key to the transformational process of the disease. Ygalo® selectively targets MM cells through aminopeptidase-driven accumulation, where in vitro experiments show a 50-fold enrichment of alkylating metabolites in MM cells. The enrichment results in selective cytotoxicity (increased on-target potency and decreased off-target toxicity), overcomes resistance pathways of existing myeloma treatments (including alkylators) and demonstrates strong anti-angiogenic properties.

## Ygalo® in clinical development

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, Ygalo® is being studied in three clinical trials for the treatment of multiple myeloma. The current studies are HORIZON, OCEAN and ANCHOR. A fourth study, BRIDGE in RRMM patients with impaired renal function will be initiated during Q3 this year to further investigate Ygalo® in multiple myeloma.

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

Ygalo® 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, three clinical studies are ongoing with Ygalo®.

**HORIZON** is a Phase II study that studies the effect of Ygalo® in late-stage RRMM patients with few or no remaining established treatment options. Updated interim data from this study are presented at EHA in June 2018.

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

In the **ANCHOR** study, Ygalo® will be 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 Ygalo® can be used in combination with these drugs. In addition, the results could open up for the use of Ygalo® in earlier lines of treatment.

### About Oncopeptides

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

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

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