Oncopeptides AB (Nasdaq Stockholm: ONCO) announced today that they will present results from two clinical studies at ASH in Atlanta, US: Final data from the phase II study called O-12-M1 and interim data from the ongoing phase II study called HORIZON. Both studies target late-stage patients with Relapsed Refractory Multiple Myeloma (RRMM).

The abstracts submitted for presentation can be found at www.oncopeptides.se/Investors & media/Presentations with the following headings:

**O-12-M1** – “First report on median overall survival (OS) and improved median progression free survival (PFS) in a completed Phase IIa Study of melflufen in advanced RRMM”.

**HORIZON** – “Melflufen therapy for Relapsed Refractory Multiple Myeloma (RRMM) patients Refractory to Daratumumab and/or Pomalidomide; a report on early efficacy”.

**CEO comments on O-12-M1**

“At ASH in December 2017, we will present the final data – that will feature in the clinical study report - from O-12-M1 in late-stage relapsed refractory multiple myeloma patients. Please note that the data in the abstract made public today is still interim, due to submission timelines for ASH. In the abstract, the median overall survival (OS) of 20.7 months and the median progression-free survival (PFS) of 5.1 months are improvements compared with the previously reported figures of 18.2 months and 4.3 months respectively. The improvement in reported PFS for Ygalo® is relevant since PFS is the primary end-point in our clinical phase III study OCEAN. The better the inherent PFS is for Ygalo®, the lower the outcome-risk is in OCEAN. We are looking forward to ASH in December when the final data will be presented. The data from O-12-M1 provides us with an increased degree of comfort regarding a positive clinical outcome in our pivotal study OCEAN” said Jakob Lindberg, CEO of Oncopeptides.

**About the O-12-M1 study**

Patient enrolment in the study was completed in December 2016. In total, 45 patients were treated with a combination of melflufen+dexamethasone in the phase II portion of the study. The median number of prior lines of therapy was four. Included patients selected had been treated with immunomodulatory drugs (IMiDs) and proteasome inhibitors (PIs), and had to be resistant to the last line of treatment defined as disease progression while on therapy or within 60 days of last dose. Of the treated patients, 62% were double refractory (IMiD + PI) and 36% were triple refractory (2 PIs+1 IMiD or 1 PI + 2 IMiDs). Patients were treated with a median of 5 cycles of Ygalo® and the median treatment duration was 17 weeks.

**Conclusions regarding O-12-M1**

Treatment with Ygalo® (melflufen), a peptidase-potentiated alkylator, shows long-term benefit in late-stage RRMM patients where conventional therapies have failed. Both the median PFS and OS in this heavily pre-treated
population, with limited remaining treatment options, are encouraging, standing at 5.1 months and 20.7 months, respectively. The long median OS of 27.2 months in patients that only achieved stable disease (SD) as best response will be studied further in the ongoing studies OCEAN (phase III) and HORIZON (phase II). Treatment-related hematologic toxicity was common, but non-hematologic adverse events (AEs) were infrequent.

**CEO comments on HORIZON**

“In the HORIZON trial, we are studying patients that are very advanced in their disease. They have been treated with both IMiDs and PIs, become refractory or non-responsive to these treatments, and after that also become refractory to later stage treatment with pomalidomide and/or daratumumab. The overall response rate (ORR) of 30% in this patient population, with a high unmet medical need, is encouraging and we look forward to presenting the updated data at ASH” said Jakob Lindberg, CEO of Oncopeptides.

**About the HORIZON study**

The study recruitment is ongoing. The abstract data is based on a data cut-off dated 27th of July 2017 when ten patients had completed at least one cycle of treatment (20 patients had been dosed). The RRMM patients enrolled in the study are refractory to pomalidomide and/or daratumumab and have had at least 2 prior lines of therapy including IMiDs and PIs. The median number of prior therapies was 6 and 70% of patients were refractory to both pomalidomide and daratumumab (in addition to failing earlier lines of therapy with IMiDs and PIs). The data will be updated at the ASH conference in December 2017.

**Conclusions regarding HORIZON**

This is a patient population with few remaining treatment options. The efficacy results in this early interim analysis are encouraging with an ORR of 30% and a clinical benefit rate (CBR) of 40% with a good safety and tolerability profile.

**About Ygalo®**

Ygalo® is a next generation alkylator compound that targets cancer cells through a mechanism called peptidase potentiation. In cell culture studies, traditional alkylators target cancer cells (which treats the disease) and bone marrow cells (which causes side effects) equally well. In contrast, Ygalo® targets the cancer cells 50x better than the bone-marrow cells.

**Ygalo® in clinical development**

Ygalo® has been used to treat late-stage RRMM patients in both phase I and phase II clinical studies with favorable results. Currently, Ygalo® is being studied in three clinical trials for the treatment of multiple myeloma. The current studies are O-12-M1, HORIZON and OCEAN. A fourth study, ANCHOR, will be initiated towards the end of 2017 to further investigate Ygalo® in multiple myeloma as part of combination therapies.

**About Multiple Myeloma**

Multiple myeloma is a hematological cancer of the B-cells (antibody producing cells) with no cure. Currently, the median overall survival is roughly 5 years and improving (Source: National Cancer Institute).

Today, approximately 170,000 patients live with multiple myeloma in the EU and the US while 57,000 patients get diagnosed and 26,000 patients die from the disease annually (Source: American Cancer Society, Global Data 2015 and National Cancer Institute). The underlying increase in number of multiple myeloma patients is slightly more than 1% per year where an aging population is the main reason for growth. However, the growth in late-stage multiple myeloma patients, that is the focus area for Ygalo®, is more than 10% per year due to improvements in
earlier lines of therapy, i.e. more patients survive the first years with multiple myeloma and become late-stage, multi-refractory patients with a significant medical need for further treatment options.

**Treating Multiple Myeloma**

Multiple myeloma is mainly treated through five different treatment modalities – alkylators, CD-38 binding antibodies, IMiDs, proteasome inhibitors and steroids. Due to the high mutation frequency of myeloma cells, patients have several different active cancers (cancer cell clones) at the same time with different protein expression patterns. Because of this heterogeneity of the disease in each patient, broad spectrum agents such as alkylators, IMiDs, proteasome inhibitors and steroids are the back-bone in its treatment. In the case of the new targeted agents, they will predominantly be used in combination with broad spectrum agents to ensure that all the patient’s cancer cells are appropriately treated. Immuno-oncological compounds have so far had limited success in the treatment of the disease.

**About Oncopeptides**

Oncopeptides is a research and development stage pharmaceutical company developing drugs for the treatment of cancer. Since the founding of the company, the focus has primarily been on the development of the lead product candidate Ygalo®, an innovative, peptidase-potentiated alkylator intended for effective and focused 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 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.

Visit [www.oncopeptides.se](http://www.oncopeptides.se) for more information.

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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 November 1, 2017 at 14.00 a.m. (CET).