

Oncopeptides presented interim data from the ongoing phase II study HORIZON with Ygalo® (melflufen) at the 59th Annual Meeting of ASH today

Stockholm – December 9th, 2017 - Oncopeptides AB (Nasdaq Stockholm: ONCO) announced today that interim data was presented at the American Society of Hematology from the ongoing phase II study HORIZON, treating late-stage patients with Relapsed Refractory Multiple Myeloma (RRMM) that are also refractory to pomalidomide and/or daratumumab.

CEO comments on HORIZON

“In the HORIZON trial, we are studying Ygalo® in patients that are advanced in their myeloma disease with very poor prognosis and no established treatment options. These patients are suffering from disease progression in conjunction with therapy, have stopped responding to lenalidomide and proteasome inhibitors, and have become refractory to rescue treatment with pomalidomide and/or daratumumab. It is therefore very encouraging to see an overall response rate (ORR) of 27% together with a good initial depth of response when treating these patients with Ygalo®” said Jakob Lindberg, CEO of Oncopeptides.

The poster presented at ASH can be found at: www.oncopeptides.se/presentations/ASH

Conference call for investors, analysts and the media

Jakob Lindberg, CEO at Oncopeptides will summarize impressions from the ASH Annual Meeting and will review and comment on relevant data from the conference including a summary of survival data from the O-12-M1 study and interim data from the ongoing HORIZON study.

Time: Wednesday, 13th December 2017, at 14.30 (CET).

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The conference call will also be streamed via a link on the website: www.oncopeptides.se and the presentation will be available on Oncopeptides website after completion of the telephone conference.

About the HORIZON study

The study recruitment is ongoing. The RRMM patients enrolled in the study are refractory to pomalidomide and/or daratumumab and have had at least two prior lines of therapy including immunomodulatory drugs (IMiDs) and proteasome inhibitors (PIs). The interim data presented at the ASH Annual Meeting are based on a data cut-off dated November 13th including 38 patients treated with a median of six prior lines of therapy and a median time from initial diagnosis of 6.3 years. 97% of patients had disease progression while on therapy or within 60 days of last dose at time of inclusion in the study (one patient had disease progression 62 days after the last dose), 86% were double-refractory to an IMiD:s and a PI, 100% were refractory to pomalidomide or daratumumab, and 62% of patients were refractory to both pomalidomide and daratumumab (see table next page).

Characteristics of prior lines of therapy (N=38)

Characteristics	Number of patients, N (%)
Double-refractory (1 IMiD + 1 PI)	32 (86)
Last line refractory (progressed while on therapy or within 60 days of last dose)*	36 (97)
Pomalidomide or daratumumab refractory	38 (100)
Pomalidomide refractory	35 (96)
Daratumumab refractory	26 (68)
Pomalidomide and daratumumab refractory	23 (62)
Alkylator exposed	34 (89)
Alkylator refractory	21 (57)

*One patient had incomplete data. One patient had PD 62 days after the last dose

Conclusions regarding HORIZON

The study continues to develop positively in this heavily pretreated patient group with few remaining treatment options. The efficacy results in this interim analysis are encouraging with an ORR of 27% and a clinical benefit rate (CBR) of 33% 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 Enhanced Cytotoxics (PEncs). In cell culture studies, traditional alkylators target the cancer cells (which treats the disease) and also bone marrow cells (which causes side effects) to an equal degree. In contrast, Ygalo® targets the myeloma 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 early 2018 to further investigate Ygalo® as part of combination therapies in multiple myeloma.

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 US).

Today, approximately 170,000 patients live with multiple myeloma in the EU and the US while 57,000 patients are newly 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 the 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, which 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 Enhanced Cytotoxics (PEncs) 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 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 December 9, 2017 at 15.00 p.m. (CET).