

## Oncopeptides announces 26% Overall Response Rate of melflufen in triple-class refractory multiple myeloma patients from the pivotal HORIZON study

STOCKHOLM — March 26, 2020 — Oncopeptides AB (Nasdaq Stockholm: ONCO) announces today the final topline results from the pivotal phase 2 HORIZON study evaluating melflufen in relapsed refractory multiple myeloma (RRMM) patients. These results will form the basis for the upcoming NDA for accelerated approval in the US. The application is on track for a submission to the FDA at the end of Q2 2020. Oncopeptides will host a webcast today to provide an update on the final study results at 14.00 (CET).

The final HORIZON results represent an Overall Response Rate (ORR) improvement for triple-class refractory RRMM patients compared to the interim data presented at the American Society of Hematology (ASH) meeting in December 2019. The HORIZON results show a good efficacy and safety profile for melflufen in difficult to treat RRMM patients. The final HORIZON data reinforce Oncopeptides' view that melflufen could play an important role in the treatment of patients with RRMM.

### Primary end-point results

Primary End-Point	Investigator Assessed January 14 <sup>th</sup>	IRC January 14 <sup>th</sup>	Incl. unconfirmed responses at time of data-cut*
ORR in the ITT population (n=157)	29%	30%	31% (inv. and IRC)
ORR in triple-class refractory patients (n=119)	26%	26%	27% (inv. and IRC)
ORR in patients with Extramedullary Disease (EMD, n=55)	24%	27%	NA

\*Two unconfirmed responders on January 14<sup>th</sup> have later been confirmed

- Data has also been reviewed by the independent review committee (IRC)
- The safety profile was consistent with previous reports from the HORIZON study with primarily hematologic Adverse Events (AE) and a low incidence of non-hematologic AEs
- Full results will be disclosed in a future peer-reviewed publication

### Jakob Lindberg, CEO comments:

“The presentation of final data from our pivotal HORIZON study, with competitive results in triple-class refractory myeloma patients, represents the most important milestone for Oncopeptides to date. These data confirm that melflufen has a good efficacy and safety profile in triple-class refractory myeloma patients – a fast-growing patient population with significant unmet medical need and lack of approved treatments. The safety profile was consistent with previous melflufen studies with good tolerability and a low rate of non-haematological adverse events. We firmly believe that melflufen has the potential to become an important treatment option for patients with relapsed refractory multiple myeloma. Study physicians and clinical sites have been immensely supportive and with their help we are on schedule to submit the NDA for accelerated approval end of Q2 2020.”

“Furthermore, with the strong final results from HORIZON our Peptide-Drug Conjugate (PDC) platform has been validated. In today’s webcast we will describe the PDC pipeline development to date and the possibilities this gives us”, concludes Jakob Lindberg.

**Oncopeptides will host a webcast today to present the data and provide a general clinical update at 14.00 (CET) that can be followed via the link:**

<https://tv.streamfabriken.com/oncopeptides-press-conference>

**Participants who would like to ask questions can use the telephone numbers below:**

Sweden: + 46 8 50558358

Europe: + 44 3333009269

USA: + 1 8446251570

**The presentation can be found at:**

[www.oncopeptides.com](http://www.oncopeptides.com) / Investor Relations / Presentations / Presentation webcast HORIZON Topline results /

**For more information, please contact:**

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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 March 26, 2020 at 09.30 (CET).

### **About the OP-106 HORIZON study**

In the pivotal phase 2 HORIZON study 157 multiple myeloma patients have been enrolled and evaluated. The study was fully recruited in October 2019 and the final data cut was made on January 14<sup>th</sup>. The patients in the study are refractory to pomalidomide and/or daratumumab after failing on immunomodulatory drugs (IMiDs) and proteasome inhibitors (PIs). The HORIZON study population includes subgroups of patients who were triple-class refractory and/or had EMD and/or had cytogenetic high-risk features.

### **About melflufen**

Melflufen (INN melphalan flufenamide) is a first-in-class anti-cancer peptide-drug conjugate that rapidly delivers an alkylating payload into tumor cells. Melflufen is rapidly taken up by myeloma cells due to its high lipophilicity and is immediately cleaved by peptidases to deliver an entrapped hydrophilic alkylator payload. Peptidases play a key role in protein homeostasis and feature in cellular processes such as cell-cycle progression and programmed cell death. In vitro, melflufen is 50-fold more potent in myeloma cells than the alkylator payload itself due to the increased intracellular alkylator concentration. 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 diseases. The company is focusing on the development of the lead product candidate melflufen, a first-in-class anti-cancer peptide-drug conjugate that rapidly delivers an alkylating payload into

tumor cells. Melflufen is in development as a new treatment for the hematological malignancy multiple myeloma and is currently being tested in multiple clinical studies including the pivotal phase 2 HORIZON study and the ongoing phase 3 OCEAN study. Oncopeptides' headquarters is in Stockholm, Sweden with its U.S. headquarters in Boston, Mass. The company is listed in the Mid Cap segment on Nasdaq Stockholm with the ticker ONCO.

More information is available on [www.oncopeptides.com](http://www.oncopeptides.com).