

The Lancet Haematology Publishes Results from Oncopeptides' Multicenter, International Phase 1/2 Study (O-12-M1)

Stockholm — March 24, 2020 — Oncopeptides AB (Nasdaq Stockholm: ONCO) announced today the publication of results from its O-12-M1 study, melflufen plus dexamethasone in relapsed/refractory multiple myeloma (RRMM), a multicenter, international, open-label, phase 1/2 study in [The Lancet Haematology](#). Melflufen is a first-in-class anti-cancer peptide-drug conjugate currently in development by Oncopeptides with activity in a variety of cancers. Current clinical development is focused on the treatment of RRMM.

In the O-12-M1 study, heavily pre-treated RRMM patients with a median of four prior lines of therapy (2-14), were dosed with 40 mg of melflufen administered intravenously once every 28 days in combination with weekly dexamethasone. Patients receiving melflufen plus dexamethasone experienced an overall response rate (ORR) of 31%, a median duration of response (DOR) of 8.4 months, and median overall survival (OS) of 20.7 months.

“We are excited to share with the scientific community these findings from our phase 1/2 study of our peptide-drug conjugate, melflufen. The results published today served as the foundation of our broad clinical development program,” said Klaas Bakker, Chief Medical Officer of Oncopeptides. “We recognize the significant unmet needs of patients with RRMM who currently have few available treatment options and are in desperate need of well-tolerated treatments with the potential to overcome cancer resistance patterns. I would like to thank all patients and their care partners for participation in this study.”

Additional findings from the study include:

- The median progression-free survival was 5.7 months
- The most common adverse events were thrombocytopenia (62%) and neutropenia (58%), while grade 3 or higher non-haematological toxicities were infrequent
- Melflufen plus dexamethasone showed activity regardless of previous treatment failure, including in patients with disease refractory to alkylators

More information about the O-12-M1 study is available on www.oncopeptides.com.

For more information, please contact:

Rein Piir, Head of Investor Relations at Oncopeptides

E-mail: rein.piiir@oncopeptides.com

Cell phone: +46 70 853 72 92

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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 cancer multiple myeloma and is currently being evaluated 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 U.S. headquarters in Boston, Mass. The company is listed in the Mid Cap segment on Nasdaq Stockholm with the ticker ONCO.