

Oncopeptides announces new clinical and preclinical melflufen data at the upcoming European Hematology Association meeting

STOCKHOLM — May 14, 2020 — Oncopeptides AB (Nasdaq Stockholm: ONCO) announces today that data from the pivotal phase 2 study HORIZON, and additional clinical and preclinical data that further evaluate the therapeutic peptide-drug conjugate platform, have been accepted by the European Hematology Association (EHA). The abstracts are now published online.

Melflufen (melphalan flufenamide) is a first-in-class anticancer peptide-drug conjugate that rapidly delivers an alkylating payload into tumor cells. Melflufen is in late stage clinical development for a potential treatment of patients with relapsed refractory multiple myeloma (RRMM).

“The primary read-out of the HORIZON-data represents an important milestone for Oncopeptides. They lay the ground for the New Drug Application to the FDA, seeking accelerated approval for intravenous melflufen in combination with dexamethasone, says Klaas Bakker, CMO of Oncopeptides. “Melflufen could provide a novel treatment option with a unique mechanism of action for a group of myeloma patients with a particularly poor prognosis”.

Below is a brief description of the abstracts that have been accepted by the European Hematology Association (EHA). The full EHA abstract book can be found on www.ehaweb.org.

1. HORIZON (OP-106): Melflufen plus dexamethasone in relapsed/refractory multiple myeloma (RRMM) refractory to pomalidomide and/or an anti-CD38 monoclonal antibody – primary and subgroup analysis. Final Abstract Code: EP945. First author: Paul G Richardson.

The primary read-out of the data from the pivotal, phase II study HORIZON demonstrates clinical efficacy and a manageable safety profile of the peptide-drug conjugate melflufen in combination with dexamethasone in patients with RRMM, including patients with high-risk features and triple-class-refractory disease.

2. HORIZON (OP-106): An exploratory analysis of time to next treatment in patients with relapsed/refractory multiple myeloma who received melflufen plus dexamethasone. Final Abstract Code: EP1029. First author: Maria-Victoria Mateos

The abstract includes a time to next treatment analysis: The sub-analysis of the HORIZON clinical study is the first to provide important insights on time to subsequent treatment in patients with advanced RRMM (medium 5 lines of previous lines).

3. LIGHTHOUSE (OP-108): A phase 3 study of melflufen in combination with daratumumab versus daratumumab in patients with relapsed/refractory multiple myeloma. Final Abstract Code: PB2018. First author: Maria-Victoria Mateos

The planned randomized phase 3 trial LIGHTHOUSE will study the impact of melflufen, dexamethasone and subcutaneous daratumumab compared with subcutaneous daratumumab alone. The results will be important to confirm the preliminary efficacy, safety and tolerability results from phase 1/2 ANCHOR study, combining melflufen, dexamethasone and daratumumab supporting further regulatory milestones for Oncopeptides

4. Adverse event and outcome patterns in patients with advanced multiple myeloma in the US

Final Abstract Code: PB2039. First author: Joshua Richter

This real-world data study provides evidence, that albeit introduction of additional treatment options for patients with advanced multiple myeloma, their prognosis remains poor and the need for additional treatment options are high

5. Melflufen is a highly effective anti-neoplastic agent in bortezomib-resistant multiple myeloma models.

Final Abstract Code: EP915. First author: Konstantin Byrgazov

Melflufen is more efficacious in bortezomib-resistant myeloma cell lines than in their bortezomib-naive parental cells in vitro. Bortezomib-resistant myeloma cells lines overexpress Aminopeptidase B encoded by RNPEP gene, and myeloma patients with high RNPEP expression have shorter PFS on bortezomib-containing therapies.

6. Melflufen efficacy in multiple myeloma with TP53 aberrations. Final Abstract Code: EP903. First author:

Ana Slipicevic.

Melflufen can trigger myeloma cell death regardless of cells TP53 status and overcome the p53-deficiency-mediated melphalan resistance. Melflufen response rate in the del 17p patient subpopulation from the phase 2-study HORIZON is comparable to the general RRMM population suggesting that melflufen might be a therapeutic option for these difficult-to-treat patients.

7. Aminopeptidase expression in multiple myeloma associates with disease progression and sensitivity to melflufen. Final Abstract Code: EP897. First author: Juho Miettinen

Aminopeptidases play a role in multiple myeloma biology. Their expression levels are dysregulated during disease progression, and majority are increased in RRMM compared to NDMM patients. Aminopeptidases LAP3 and TPP2 are identified as prognostic markers in myeloma patients, and inhibition of aminopeptidases reduces myeloma cell viability in vitro. Melflufen, an aminopeptidase substrate, is a highly efficient anticancer agent in myeloma cells resistant to other alkylators, bortezomib and selinexor.

For more information, please contact:

Klaas Bakker, MD, PhD, Chief Medical Officer of Oncopeptides

E-mail: klaas.bakker@oncopeptides.com

Cell: +44 7818 523903

Fredrik Lehmann, EVP Research and CMC at Oncopeptides

E-mail: Fredrik.lehmann@oncopeptides.com

Phone: +46 8 615 20 40

Rein Piir, Head of Investor Relations at Oncopeptides
E-mail: rein.piid@oncopeptides.com
Cell: +46 70 853 72 92

This information was submitted for publication at 15.00 CET May 14, 2020.

About melflufen

Melflufen (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.

More information is available on www.oncopeptides.com.