Pepaxto® (melphalan flufenamide) – Withdrawal from the U.S. market

- On October 22, 2021, Oncopeptides announced the withdrawal of Pepaxto (melphalan flufenamide) following a clinical trial (OCEAN) that demonstrated an overall survival in the intention to treat population with a hazard ratio of 1.104.

- In July 2021, the FDA alerted patients and health care providers that the study evaluating Pepaxto with dexamethasone to treat patients with multiple myeloma showed an increased risk of death.

    - At that time, the FDA encouraged health care providers to review patients' progress on Pepaxto and discuss the risks and benefits of treatment.
    - The FDA also required Oncopeptides to suspend enrollment in the OCEAN trial and any other ongoing Pepaxto clinical trials.

- The trial compared Pepaxto with low-dose dexamethasone to Pomalyst® (pomalidomide) with low-dose dexamethasone in patients with relapsed or refractory (resistant) multiple myeloma following 2-4 lines of prior therapy and in patients who were resistant to Revlimid® (lenalidomide) in the last line of therapy.

    - There were 495 randomized patients included in the efficacy analysis.
    - For overall survival (OS), there were 117/246 (48%) deaths in the Pepaxto investigational arm and 108/249 (43%) deaths in the Pomalyst control arm.
    - The hazard ratio of the Pepaxto arm vs. Pomalyst arm was 1.104 (95% CI: 0.846, 1.441) indicating a detriment in survival with Pepaxto vs. Pomalyst.
    - The median OS with Pepaxto was 19.7 months (95% CI: 15.1, 25.6) vs. 25.0 months (95% CI: 18.1, 31.9) with Pomalyst. The median follow-up for survival was 19.1 months.

- The FDA does not consider that the phase 3 OCEAN study meets the criteria of a confirmatory study. Oncopeptides believes that the OCEAN data are scientifically meaningful and that the findings warrant further evaluation.

- Oncopeptides will work together with the FDA to continue to make the drug available for patients currently treated with Pepaxto.

- In February 2021, the FDA approved Pepaxto under accelerated approval for use in combination with dexamethasone to treat adult patients with relapsed or refractory multiple myeloma who have received at least four prior lines of therapy and whose disease was refractory to at least one proteasome inhibitor, one immunomodulatory agent, and one CD38-directed monoclonal antibody. Oncopeptides was required to conduct the OCEAN trial as a post-approval requirement under the accelerated approval program.