The FDA approves melphalan flufenamide for patients with relapsed or refractory myeloma

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The U.S. Food and Drug Administration (FDA) has approved melphalan flufenamide (PEPAXTO®), also known as melflufen, in combination with dexamethasone, for the treatment of adult patients with relapsed or refractory myeloma, who have received at least four prior lines of therapy and whose disease is refractory to at least one proteasome inhibitor, one immunomodulatory agent, and one CD38-directed monoclonal antibody.

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This drug is the first anticancer peptide-drug conjugate approved by the FDA. The product has been granted accelerated approval based on the **phase 2 HORIZON study** in relapsed or refractory myeloma patients.

The HORIZON study evaluating intravenous melflufen in combination with dexamethasone, included heavily pre-treated patients with a poor prognosis. This multi-center single arm study evaluated 157 patients with relapsed or refractory multiple myeloma, of whom 97 were triple-class refractory and had received at least four prior lines of treatment. The Overall Response Rate for the patients within this group of patients with refractory multiple myeloma was 23.7% and the Median Duration of Response was 4.2 months. Furthermore, melflufen in combination with dexamethasone demonstrated activity in a subset of patients with Extra Medullary Disease (41%), an aggressive and resistant disease associated with a poor prognosis.