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ONO Submits Manufacturing and Marketing Approval Application in Japan for "Carfilzomib (ONO-7057)", a Proteasome Inhibitor, in Relapsed or Refractory Multiple Myeloma

ONO PHARMACEUTICAL CO.,LTD. (Osaka, Japan; President, Representative Director and CEO: Gyo Sagara; hereinafter, "ONO") today announced the submission of Manufacturing and Marketing Approval Application for carfilzomib for Injection "(ONO-7057)," which is a proteasome inhibitor, to seek an indication for relapsed or refractory multiple myeloma.

Multiple myeloma results from an abnormality of plasma cells, usually in the bone marrow and there are nearly 14,000 patients [1)] in Japan. Several regimens for multiple myeloma are currently available to patients; however, the disease relapses and progresses and eventually becomes no longer responding to therapies, also known as refractory disease. Additionally, adverse drug reactions and co-morbid conditions have been reported following long-term treatment, making continued treatment a challenge. The development of new therapeutic options for multiple myeloma are needed.

Carfilzomib is in a class of drugs called proteasome inhibitors that ONO in-licensed for development and commercialization in Japan from Onyx Pharmaceuticals, Inc., an Amgen subsidiary, in the U.S. in September 2010. Proteasome, an intra-cellular enzyme complex, functions to mediate degradation of polyubiquitinated proteins and control proliferation and differentiation of cells, as well as functional cell-death. Carfilzomib inhibits certain proteasome activity, thereby inducing functional cell-death of myeloma.

In July 2012, the U.S. Food and Drug Administration (FDA) granted accelerated approval of carfilzomib as a single agent for the treatment of patients multiple myeloma who have received at least two prior therapies including bortezomib and an immunomodulatory agent and have demonstrated disease progression on or within 60 days of completion of the last therapy, and the drug has been already launched. Also, in July 2015, the FDA approved carfilzomib in combination with lenalidomide and dexamethasone for the treatment of patients with relapsed multiple myeloma who have received one to three prior lines of therapy. In January 2015, the Marketing Authorization Application for carfilzomib was submitted in Europe; the application was granted accelerated assessment. In Japan, the Ministry of Health, Labour and Welfare designated carfilzomib as an orphan drug with a proposed indication for the treatment of "relapsed or refractory multiple myeloma" in August 20, 2015.

1) Vital Statistics and Patients Survey, 2011 (Statistics and Information Department, Minister's Secretariat, Ministry of Health, Labour and Welfare).