ONO PHARMACEUTICAL CO.,LTD.

September 7, 2016

ONO Submits Supplemental New Drug Application in Japan for "KYPROLIS® for Intravenous Injection 10 mg and 40 mg", a Proteasome Inhibitor, for Relapsed or Refractory Multiple Myeloma

ONO PHARMACEUTICAL CO., LTD. (Osaka, Japan; President, Representative Director and CEO: Gyo Sagara; hereinafter, "ONO") today announced that it submitted a supplemental New Drug Application (sNDA) on August 25 for "KYPROLIS® for Intravenous Injection 10 mg and 40 mg" (Generic name: carfilzomib, hereinafter, "Kyprolis"), a proteasome inhibitor, which was launched in Japan on August 31 for the treatment of patients with relapsed or refractory multiple myeloma.

The sNDA is based on data from the global Phase 3 study (2011-003, ONO-7057-03, or ENDEAVOR) of Kyprolis plus dexamethasone versus bortezomib plus dexamethasone. On July 4, 2016, Kyprolis was granted a manufacturing and marketing approval in combination with lenalidomide and dexamethasone at a dosage of 20 mg/m^2 in Cycle 1 on Day 1 and 2, and escalate to 27 mg/m^2 thereafter. The sNDA is to seek an expanded indication for Kyprolis in combination with dexamethasone at a dosage of 20 mg/m^2 in Cycle 1 on Day 1 and 2, and escalate to 56 mg/m^2 thereafter.

Multiple myeloma results from an abnormality of plasma cells, usually in the bone marrow and there are about 18,000 patients* 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 is needed.

Kyprolis is in a class of drugs called proteasome inhibitors. ONO in-licensed Kyprolis for development and commercialization in Japan from U.S.-based Onyx Pharmaceuticals, Inc., now an Amgen subsidiary, 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. Kyprolis inhibits certain proteasome activity, thereby inducing functional cell-death of myeloma.

In the US, the U.S. Food and Drug Administration (FDA) granted accelerated approval of Kyprolis as a single agent in July 2012 for the treatment of patients with multiple myeloma. Kyprolis is currently used; 1) as a single agent for the treatment of patients with relapsed or refractory multiple myeloma who have received one or more lines of therapy, and 2) in combination with dexamethasone or with lenalidomide plus dexamethasone for the treatment of patients with relapsed or refractory multiple myeloma who have received one to three lines of therapy. In Europe, the Marketing Authorization Application for Kyprolis was approved in November 2015 in combination with lenalidomide and dexamethasone for the treatment of adult patients with multiple myeloma who have received at least one prior therapy. Kyprolis is currently used in combination with either

lenalidomide and dexamethasone or dexamethasone alone for the treatment of adult patients with multiple myeloma who have received at least one prior therapy.

*: Vital Statistics and Patients Survey, 2014 (Statistics and Information Department, Minister's Secretariat, Ministry of Health, Labour and Welfare).

ONO PHARMACEUTICAL CO., LTD.
Corporate Communications
public_relations@ono.co.jp