Press Release

First Patient Dosed in Phase 3 MORPHO Trial Evaluating Gilteritinib as Maintenance Therapy Following Hematopoietic Stem Cell Transplant in Patients with FLT3 Mutation-positive Acute Myeloid Leukemia

TOKYO – August 22, 2017 – Astellas Pharma Inc. (TSE: 4503, President and CEO: Yoshihiko Hatanaka, "Astellas") announced today that the first patient was dosed in the registrational Phase 3 MORPHO trial of gilteritinib, the fourth Phase 3 trial underway in the gilteritinib clinical development program. The MORPHO trial is a randomized, double-blind, placebo-controlled, multi-center trial that compares gilteritinib to placebo as maintenance therapy over a period of two years following hematopoietic stem cell transplant (HCT) in patients with FLT3 internal tandem duplication (ITD) mutation-positive (FLT3/ITD+) acute myeloid leukemia (AML) and in remission after induction therapy. The primary endpoint is relapse-free survival (RFS), and the study is being conducted in collaboration with the Blood and Marrow Transplant Clinical Trials Network (BMT CTN).

"We know that FLT3+ AML patients face potentially worse outcomes than those with other mutations, and while some patients may experience remission following a stem cell transplant, many unfortunately relapse," said trial investigator and BMT CTN Study co-chair Mark J. Levis, M.D., Ph.D., of the Sidney Kimmel Comprehensive Cancer Center at Johns Hopkins University. "Given this reality, it is exciting to study gilteritinib in patients following a stem cell transplant."

Gilteritinib has demonstrated inhibitory activity against FLT3 ITD as well as FLT3 tyrosine kinase domain (TKD), two common types of FLT3 mutations that are seen in approximately one third of patients with AML. Further, gilteritinib has also demonstrated inhibition of AXL, which is reported to be associated with therapeutic resistance.

AML is a cancer that impacts the blood and bone marrow and is most commonly experienced in older adults. According to the American Cancer Society, in 2016 there were an estimated 21,000 new cases of AML diagnosed in the United States and about 10,600 cases resulted in death.

"The initiation of the MORPHO trial is another significant milestone for Astellas and for patients as therapeutic options can be very limited for this FLT3+ AML population," said Steven Benner, M.D., senior vice president and global therapeutic area head, oncology development, Astellas. "We are committed to patients with FLT3+ AML and currently have underway four Phase 3 trials to explore the potential benefit of gilteritinib for patients suffering from such an aggressive form of blood cancer characterized by both genetic and resistance mutations."

Astellas is currently investigating gilteritinib in various AML patient populations through several Phase 3 trials, including the registrational ADMIRAL trial in relapsed/refractory FLT3+ AML.

The safety and efficacy of the agent discussed herein are under investigation and have not been established. There is no guarantee that the agent will receive regulatory approval and become commercially available for the uses being investigated. Information about pharmaceutical products (including products currently in development) which is included in this press release are not intended to constitute an advertisement or medical advice.

About the MORPHO Study
The Phase 3 MORPHO Study is a two-arm, randomized, double-blind, placebo-controlled, multi-center trial in 346 patients with a diagnosis of AML harboring a FLT3/ITD mutation. Participants must be in first complete remission prior to transplant, as defined by less than five percent blasts in the bone marrow (BM) with no morphologic characteristics of acute leukemia in the BM with no evidence of extra-medullary leukemia. After undergoing transplantation, participants will be randomized to receive gilteritinib (120 mg) or placebo beginning after the time of engraftment for a two-year period. Participants will be stratified according to: 1) conditioning regimen intensity (myeloablative vs. reduced intensity), 2) time from HCT to randomization (30-60 days vs. 61-90 days) and 3) presence or absence of minimal residual disease from the most recent pre-registration BM aspirate. The primary endpoint of the trial is RFS. The study is being conducted in countries across North America, Europe and the Asia-Pacific region, including Japan. For more information about this trial, go to www.clinicaltrials.gov trial identifier NCT02997202.

About Gilteritinib
Gilteritinib was discovered through a research collaboration with Kotobuki Pharmaceutical Co., Ltd., and Astellas has exclusive global rights to develop, manufacture and potentially commercialize gilteritinib. Astellas classifies highly prioritized research projects as “Fast Track,” meaning research and development time is minimized through the focused investment of both capabilities and resources. Gilteritinib was designated as our first Fast Track project.

About Astellas
Astellas Pharma Inc., based in Tokyo, Japan, is a company dedicated to improving the health of people around the world through the provision of innovative and reliable pharmaceutical products. We focus on Urology, Oncology, Immunology, Nephrology and Neuroscience as prioritized therapeutic areas while advancing new therapeutic areas and discovery research leveraging new technologies/modalities. We are also creating new value by combining internal capabilities and external expertise in the medical/healthcare business. Astellas is on the forefront of healthcare change to turn innovative science into value for patients. For more information, please visit our website at www.astellas.com/en.

About BMT CTN
The Blood and Marrow Transplant (BMT) Clinical Trials Network (CTN) was established in October 2001 to conduct large, multi-institutional clinical trials to improve outcomes for patients who receive hematopoietic cell transplantation. The

network is funded by the National Heart, Lung, and Blood Institute (NHLBI) and the National Cancer Institute (NCI) of the National Institutes of Health.

Cautionary Notes

In this press release, statements made with respect to current plans, estimates, strategies and beliefs and other statements that are not historical facts are forward-looking statements about the future performance of Astellas. These statements are based on management's current assumptions and beliefs in light of the information currently available to it and involve known and unknown risks and uncertainties. A number of factors could cause actual results to differ materially from those discussed in the forward-looking statements. Such factors include, but are not limited to: (i) changes in general economic conditions and in laws and regulations, relating to pharmaceutical markets, (ii) currency exchange rate fluctuations, (iii) delays in new product launches, (iv) the inability of Astellas to market existing and new products effectively, (v) the inability of Astellas to continue to effectively research and develop products accepted by customers in highly competitive markets, and (vi) infringements of Astellas' intellectual property rights by third parties.

Information about pharmaceutical products (including products currently in development) which is included in this press release is not intended to constitute an advertisement or medical advice.

Contacts for inquiries or additional information:

Astellas Pharma Inc.
Corporate Communications
TEL: +81-3-3244-3201 FAX: +81-3-5201-7473