Press Release

U.S. FDA Grants Priority Review to Astellas' New Drug Application for Gilteritinib for the Treatment of Adult Patients with Relapsed or Refractory Acute Myeloid Leukemia (AML)

TOKYO – May 29, 2018 – Astellas Pharma Inc. (TSE: 4503, President and CEO: Kenji Yasukawa, Ph.D. "Astellas") announced today that the U.S. Food and Drug Administration (FDA) has accepted, with Priority Review, the company's New Drug Application (NDA) for gilteritinib for the treatment of adult patients who have relapsed or refractory (resistant to treatment) Acute Myeloid Leukemia (AML) with a FLT3 mutation as detected by an FDA-approved test. Currently, there are no FLT3-targeting agents approved for the treatment of relapsed or refractory FLT3 mutation-positive (FLT3mut+) AML.

"FLT3 mutations impact approximately 30 percent of AML patients and are often associated with poor survival outcomes. Many with this condition relapse after treatment or don't respond to currently available treatments. Simply put, they need more options," said Steven Benner, M.D., senior vice president and global therapeutic area head, Oncology Development, Astellas. "The FDA's acceptance of this NDA, with Priority Review, represents a significant milestone for gilteritinib and Astellas in our mission to help AML patients and the physicians who treat them."

The NDA is based on the ongoing Phase 3 ADMIRAL trial investigating gilteritinib for the treatment of adult patients with FLT3mut+ relapsed or refractory AML. The Prescription Drug User Fee Act (PDUFA) goal date for a decision by the FDA is November 29, 2018.

The FDA grants Priority Review designation to applications for drugs that, if approved, may offer significant improvements in the safety and effectiveness of the treatment of serious conditions when compared to standard applications. Under Priority Review, the FDA aims to take action on an application within six months of receipt, as compared to ten months under standard review.

Previously, gilteritinib was granted both Orphan Drug designation and Fast Track designation by the U.S. FDA. Gilteritinib also received Orphan Designation from the European Commission (EC) and Orphan Drug Designation from the Japan Ministry of Health, Labor and Welfare (MHLW). The MHLW also granted SAKIGAKE designation to gilteritinib for relapsed/refractory AML.

About Acute Myeloid Leukemia

Acute Myeloid Leukemia (AML) is a cancer that impacts the blood and bone marrow, and its incidence increases with age. The American Cancer Society estimates that in 2018, approximately 19,000 new patients will be diagnosed with AML in the U.S.[1] In Western Europe, there are around 13,000 new cases of AML every year.[2] In Japan, approximately 5,500 patients are diagnosed with AML each year.[3]

About Gilteritinib
Gilteritinib is an investigational compound that has demonstrated inhibitory activity against FLT3 internal tandem duplication (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 the AXL receptor in AML cell lines. Astellas is currently investigating gilteritinib in various AML patient populations through several additional Phase 3 trials. Visit AstellasAMLTrials.com to learn more about ongoing gilteritinib clinical trials.

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.

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 ADMIRAL Trial[4]
The Phase 3 ADMIRAL trial (NCT02421939) is an open-label, multicenter, randomized study of gilteritinib versus salvage chemotherapy in adult patients with FLT3 mutations who are refractory to or have relapsed after first-line AML therapy. The primary endpoints of the trial are Overall Survival (OS) and complete remission/complete remission with partial hematologic recovery (CR/CRh) rates. The study was designed to enroll 369 patients with FLT3 mutations present in bone marrow or whole blood, as determined by central lab. Subjects were randomized in a 2:1 ratio to receive gilteritinib (120 mg[5]) or salvage chemotherapy.

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. For more information, please visit our website at https://www.astellas.com/en

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.

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[1] American Cancer Society. Key Statistics for Acute Myeloid Leukemia (01-04-2018), https://www.cancer.org/cancer/acute-myeloid-leukemia/about/key-statistics.html. Last accessed 03-12-2018.
[2] Kashyap A. Decision Resources Group. Acute Myeloid Leukemia: disease landscape and forecast (10-2017).
[3] Kantar Health. Treatment Architecture: United States acute myeloid leukemia. CancerMPact (12-2017).
[4] ClinicalTrials.gov. A Study of ASP2215 Versus Salvage Chemotherapy in Patients With Relapsed or Refractory Acute Myeloid Leukemia (AML) With FMS-like Tyrosine Kinase (FLT3) Mutation (03-20-2018). https://clinicaltrials.gov/ct2/show/NCT02421939?cond=02421939&rank=1. Accessed 03-26-2018.
[5] Gorcea CM, Burthem J, Tholouli E. ASP2215 in the treatment of relapsed/refractory acute myeloid leukemia with FLT3 mutation: background and design of the ADMIRAL trial. Future Oncol (epub) 03-02-2018.