Press Release

# Astellas Receives Orphan Designation from the European Commission for Gilteritinib for the Treatment of Acute Myeloid Leukaemia

**TOKYO – January 23, 2018 –** Astellas Pharma Inc. (TSE: 4503, President and CEO: Yoshihiko Hatanaka, "Astellas") announced today that the European Commission (EC) has issued Orphan Designation to gilteritinib for the treatment of patients with acute myeloid leukaemia (AML). The decision follows a positive recommendation for Orphan Designation from the European Medicines Agency's (EMA) Committee for Orphan Medicinal Products (COMP). In Europe, an Orphan Designation is granted to a medicine that may be of significant benefit to patients with a rare condition, affecting no more than five in 10,000 people.[1]

The announcement follows the recent Orphan Drug Designation in the United States granted by the US Food and Drug Administration (FDA) to gilteritinib on July 13, 2017.[2]

"Around 13,000 people will be diagnosed with AML in Western Europe and, while AML patients constitute a small proportion of the overall population, they are faced with a life-threatening condition," said Steven Benner, M.D., Senior Vice President and Global Therapeutic Area Head, Oncology Development, Astellas. "We are grateful to the EMA for acknowledging the unique needs of patients with rare diseases, and for providing a potential path forward for gilteritinib in supporting these patients."

AML is a cancer that impacts the blood and bone marrow, and its incidence increases with age.[3] In Western Europe, there are around 13,000 new cases of AML every year.[4] In Japan, approximately 4,500 patients are diagnosed with AML each year.[5,6] The American Cancer Society estimates that in 2017 approximately 21,000 new patients will be diagnosed with AML in the United States and about 10,000 cases will result in death.[7]

**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),[8] two common types of FLT3 mutations that are seen in approximately one-third of patients with AML.[9] Further, gilteritinib has also demonstrated inhibition of the AXL receptor in AML cell lines.[8] Astellas is currently investigating gilteritinib in various FLT3 mutation-positive AML patient populations through several Phase 3 trials.[10,11] Visit www.clinicaltrials.gov 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 commercialise gilteritinib. Gilteritinib has been granted

Orphan Drug designation[2] and Fast Track Designation[12] by the U.S. FDA, and SAKIGAKE Designation by the Japan Ministry of Health, Labour and Welfare.[13]

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.

**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 prioritised 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.

**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.

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