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### **ASTELLAS ANNOUNCES PRELIMINARY PHASE 1/2 SAFETY, TOLERABILITY AND EFFICACY DATA FOR ASP2215 IN PATIENTS WITH RELAPSED OR REFRACTORY ACUTE MYELOID LEUKEMIA (AML)**

- *Results from Phase 1/2 trial investigating drug candidate for rare, aggressive blood cancer announced at American Society of Clinical Oncology annual meeting; Phase 3 trial planned for late 2015.*
- *Abstract is one of several Astellas Oncology data presentations scheduled for the ASCO annual meeting*

**TOKYO** – May 30, 2015 – Astellas Pharma Inc. (TSE: 4503) today announced that preliminary data from a Phase 1/2 trial on the safety, tolerability and efficacy of ASP2215, a selective inhibitor of FLT3/AXL, in patients with relapsed or refractory (R/R) acute myeloid leukemia (AML) were presented during an oral abstract session at the American Society of Clinical Oncology's (ASCO) annual meeting in Chicago.

ASP2215 is a receptor tyrosine kinase inhibitor of FLT3 and AXL, which are involved in the growth of cancer cells. ASP2215 demonstrated inhibitory activity against FLT3 internal tandem duplication (ITD) as well as tyrosine kinase domain (TKD), two common types of FLT3 mutations that are seen in up to one third of patients with AML. Preliminary data from the Phase 1/2 trial demonstrated a 57.5 percent overall response rate and a 47.2 percent composite Complete Response (CR) rate (CR + CR with incomplete platelet recovery + CR with incomplete hematologic recovery) in 106 patients with FLT3 mutations who received 80 mg and higher doses. Furthermore, median duration of response was 18 weeks across all doses and median overall survival was approximately 27 weeks at 80 mg and above in FLT3 mutation positive patients. A plasma inhibitory activity assay also confirmed sustained FLT3 inhibition consistently in patients receiving doses of 80 mg and above.

The ASP2215 Phase 1/2 trial design followed a 3+3 escalation and evaluated doses from 20 to 450 mg once daily. A parallel multi-dose expansion cohort was initiated based on the efficacy seen in dose escalation. A total of 198 patients were enrolled in the study: 24 in the dose escalation and 174 in the dose expansion cohorts. At the 450 mg dose, two patients reached dose-limiting toxicity (grade 3 diarrhea and ALT/AST elevation) and the maximum tolerated dose was determined to be 300 mg.

“ASP2215 demonstrated the ability to inhibit the FLT3 mutation that is seen in approximately 30 percent of AML patients,” said Mark J. Levis, M.D., Ph.D., Department of Oncology, Sidney Kimmel Comprehensive Cancer Center, Johns Hopkins University.

“At Astellas Oncology, we are focused on developing targeted therapies for hard-to-treat cancers with few therapeutic options, such as AML,” said Claire Thom, Pharm.D., senior vice president and oncology therapeutic head, Astellas Pharma Global Development, Inc. “We are very excited about these results as they indicate that ASP2215 may be a therapeutic option in this underserved patient population. We look forward to moving this candidate into Phase 3 trials to further explore the full potential of the compound for patients suffering from AML.”

A randomized Phase 3 trial of ASP2215 at 120 mg per day in relapsed and refractory AML patients is planned. For more information, visit <https://clinicaltrials.gov/ct2/show/NCT02421939>.

ASP2215 was discovered through a research collaboration with Kotobuki Pharmaceutical Co., Ltd., and Astellas has exclusive global rights to develop, manufacture and potentially commercialize ASP2215.

### **About Acute Myeloid Leukemia**

Acute myeloid leukemia is a cancer that impacts the blood and bone marrow and most commonly experienced in older adults. According to the [American Cancer Society](#), in 2015, there will be an estimated 20,830 new cases of AML diagnosed in the United States, and about 10,460 cases will result in death.

### **About Astellas**

Astellas is a pharmaceutical company dedicated to improving the health of people around the world through provision of innovative and reliable pharmaceuticals. For more information on Astellas, please visit our website at [www.astellas.us](http://www.astellas.us), follow us on Twitter at [www.twitter.com/AstellasUS](https://www.twitter.com/AstellasUS) or like our Facebook page at [www.facebook.com/AstellasUS](https://www.facebook.com/AstellasUS).

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