Press Release

Astellas Reports XOSPATA® (gilteritinib) in Combination with Azacitidine Did Not Meet Endpoint of Overall Survival in Newly Diagnosed FLT3 Mutation-Positive Acute Myeloid Leukemia Patients Ineligible for Intensive Induction Chemotherapy

TOKYO, December 21, 2020 – Astellas Pharma Inc. (TSE: 4503, President and CEO: Kenji Yasukawa, Ph.D., "Astellas") today announced that a Phase 3 trial of XOSPATA® (gilteritinib) plus azacitidine versus azacitidine alone in newly diagnosed FLT3 mutation-positive (FLT3mut+) acute myeloid leukemia (AML) patients who were ineligible for intensive induction chemotherapy did not meet its primary endpoint of overall survival at a planned interim analysis of the LACEWING trial. An independent Data Monitoring Committee recommended terminating the study for futility, concluding results are unlikely to show a statistically significant increase in overall survival. Astellas has stopped enrollment in the trial and is reviewing the results for other action as needed.

"Although we are disappointed by the primary outcome of LACEWING, we are conducting a thorough review of the data and plan to share detailed results at a later date," said Andrew Krivoshik, M.D., Ph.D., Senior Vice President and Oncology Therapeutic Area Head, Astellas. "These results do not affect other ongoing gilteritinib trials. We remain committed to our comprehensive program investigating gilteritinib across a wide range of AML patients with a positive FLT3 mutation, building on gilteritinib's earlier, positive data in patients with relapsed or refractory FLT3 mutation-positive AML."

AML, a cancer of the blood and bone marrow, is one of the most common types of leukemia in adults.[1] It has the lowest survival rate of all types of leukemia.[2] Approximately one-third of people with AML have a FLT3 mutation.[3,4] This mutation is associated with worsened disease-free survival and overall survival, and a higher risk of getting the disease more than once.[3,4,5] Among patients with FLT3mut+ AML, an estimated 30-40 percent are not candidates for intensive chemotherapy regimens because of age, performance status, and/or comorbid conditions.[6]

About the LACEWING trial

The Phase 3 LACEWING trial (NCT02752035) is an open-label, multicenter, randomized trial designed to evaluate gilteritinib plus azacitidine versus azacitidine alone in approximately 250 newly diagnosed FLT3mut+ AML patients who are ineligible for first-line intensive induction chemotherapy. The primary endpoint is overall survival defined as the time from the date of randomization until the date of death from any cause. Key secondary endpoints include event-free survival (EFS), best response, complete remission, composite complete remission and complete remission with partial hematologic recovery.

About Gilteritinib

Gilteritinib was discovered through a research collaboration with Kotobuki Pharmaceutical Co., Ltd., and Astellas has exclusive global rights to develop, manufacture and commercialize gilteritinib. Gilteritinib is available as XOSPATA® in the U.S., Japan and selected European countries, among others, for the treatment of adult patients who have relapsed or refractory FLT3mut+ AML.[6] Gilteritinib is an FMS-like tyrosine kinase 3 (FLT3) inhibitor with demonstrated activity against FLT3-ITD, a common driver mutation that presents with a high burden and poor prognosis, and FLT3-TKD mutations.[7]

About Astellas
Astellas Pharma Inc. is a pharmaceutical company conducting business in more than 70 countries around the world. We are promoting the Focus Area Approach that is designed to identify opportunities for the continuous creation of new drugs to address diseases with high unmet medical needs by focusing on Biology and Modality. Furthermore, we are also looking beyond our foundational Rx focus to create Rx+® healthcare solutions that combine our expertise and knowledge with cutting-edge technology in different fields of external partners. Through these efforts, Astellas stands on the forefront of healthcare change to turn innovative science into value for patients. 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.

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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