Press release

Astellas Announces Acceptance of XOSPATA™ (gilteritinib) for Regulatory Review by the European Medicines Agency

TOKYO, February 28, 2019 – Astellas Pharma Inc. (TSE: 4503, President and CEO: Kenji Yasukawa, Ph.D., "Astellas") announced today that the submission for a marketing authorisation application (MAA) for the oral once-daily therapy XOSPATA (gilteritinib) for the treatment of adult patients who have relapsed or refractory (resistant to treatment) acute myeloid leukaemia (AML) with a FLT3 mutation (FLT3mut+) has been accepted by the European Medicines Agency for regulatory review.

Astellas applied for and received accelerated assessment from the European Medicines Agency (EMA) for gilteritinib, which means the Committee for Medicinal Products for Human Use (CHMP) can reduce the timeframe for approval from 210 to 150 days.[1]

The MAA is based on data from the Phase 3 ADMIRAL trial investigating gilteritinib for the treatment of adult patients with FLT3mut+ relapsed or refractory AML. The full results of the ADMIRAL trial will be presented at the American Association for Cancer Research (AACR) Annual Meeting 2019, March 29 – April 3 in Atlanta, USA.

AML is a cancer that impacts the blood and bone marrow, and its incidence increases with age.[2] The incidence rate of AML is 3.7 per 100,000 per year in the European Union, resulting in an estimated 18,400 individuals diagnosed.[3]

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NOTES TO EDITORS

About gilteritinib
Gilteritinib was discovered through a research collaboration with Kotobuki Pharmaceutical Co., Ltd., and Astellas has exclusive global rights to develop, manufacture and commercialise gilteritinib.
Last year gilteritinib received an Orphan Designation from the European Commission[4] and received accelerated assessment from the EMA[1]. In both Japan and the U.S., gilteritinib was launched in December 2018 for the treatment of adult patients with relapsed or refractory FLT3mut+ AML.[5,6] Gilteritinib was previously granted Orphan Drug designation and Fast Track designation by the U.S. FDA, and Orphan Drug Designation and SAKIGAKE designation from the Japanese Ministry of Health, Labour and Welfare.[7]
Astellas is currently investigating gilteritinib in various FLT3 mutation-positive AML patient populations through several Phase 3 trials.

About the ADMIRAL Trial[8]
The Phase 3 ADMIRAL trial (NCT02421939) was an open-label, multicentre, randomised 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 enrolled 371 patients with FLT3 mutations present in bone marrow or whole blood, as determined by central lab. Subjects were randomised in a 2:1 ratio to receive gilteritinib (120 mg[9]) 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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References

[1] EMA. Accelerated assessment. Available at: https://www.ema.europa.eu/human-regulatory/marketing-authorisation/accelerated-assessment. Last accessed January 2019.
[2] Cancer Research UK. About Acute Myeloid Leukemia. Available at: https://www.cancerresearchuk.org/about-cancer/acute-myeloid-leukaemia-aml/about-acute-myeloid-leukaemia Last accessed January 2019
[3] O.Visser et al. Incidence, survival and prevalence of myeloid malignancies in Europe. European Journal of Cancer (2012) 48, 3257–3266
[4] EMA. Orphan designation. Available at: http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000029.jsp Last accessed January 2019.
[5] Japan Pharmaceutical and Medical Devices Agency (PMDA). New Drug approvals, May-October 2018. Available at: http://www.pmda.go.jp/files/000227117.pdf#page=5 Last accessed January 2019.
[6] US Food and Drug Administration. FDA approves treatment for adult patients who have relapsed or refractory acute myeloid leukemia (AML) with a certain genetic mutation. Available at: https://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm627072.htm Last accessed January 2019.
[7] Ministry of Health, Labour and Welfare - Japan. Press announcement – Priority Assessment Designation System. Available at: http://www.mhlw.go.jp/stf/houdou/0000102009.html Last accessed January 2019.
[8] ClinicalTrials.gov. A study of ASP2215 versus salvage chemotherapy in patients with relapsed refractory acute myeloid leukemia (AML) with FMS-like tyrosine kinase (FLT3) mutation (04-25-2018). https://clinicaltrials.gov/ct2/show/NCT02421939 Last accessed January 2019.
[9] 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. In Future Oncology Epub (ahead of print)