Press Release

Astellas Receives Orphan Drug Designation from the Japanese MHLW for Gilteritinib

Tokyo, March 22, 2018 - Astellas Pharma Inc. (President and CEO: Yoshihiko Hatanaka, "Astellas") today announced that the Japanese Ministry of Health, Labour and Welfare (MHLW) has granted Orphan Drug Designation to gilteritinib for the treatment of FLT3mut+ Acute Myeloid Leukemia (AML), the drug's prospective indication.

Orphan drug designation system in Japan aims to support the development of drugs for diseases that, despite there being a significant medical need for treatments, affect only a small number of patients, and for which research and development is virtually nonexistent. As stipulated in Article 77 of the Pharmaceuticals, Medical Devices, and Other Therapeutic Products Act of Japan, the designation is granted by the minister of Health, Labour and Welfare for drugs that meet the designation criteria which include the following: the number of patients who may use the drug is less than 50,000 in Japan; there is no alternative appropriate drug or treatment in Japan; high efficacy or safety is expected compared to existing products. Specific measures to support the development of orphan drugs include subsidies for research and development expenditures, prioritized consultation regarding clinical development, reduced consultation fees, tax incentives, priority review of applications, reduced application fees, and extended registration validity period[1].

AML is a cancer that impacts the blood and bone marrow, and its incidence increases with age. In Japan, approximately 5,500 patients are diagnosed with AML each year[2]. 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 will continue to develop gilteritinib to provide it to FLT3mut+ AML patients as early as possible.

(1): http://www.mhlw.go.jp/stf/seisakunitsuite/bunya/0000068484.html

(2): KantarHealth. TREATMENT ARCHITECTURE: JAPAN LEUKEMIA, ACUTE MYELOID. CancerMPact® Japan, February 2017.

About Gilteritinib

Astellas is currently investigating gilteritinib in various FLT3 mutation-positive AML patient populations through several Phase 3 trials. Visit http://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 commercialize gilteritinib. Gilteritinib has been granted Orphan Drug designation and Fast Track designation by the U.S. FDA, Orphan Designation by the European Commission and SAKIGAKE Designation by the Japan Ministry of Health, Labour and Welfare.

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 prioritized 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 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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