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## **Masitinib Receives Orphan Drug Designation for Stomach Cancer from FDA**

**AB Science SA** (NYSE Euronext – FR0010557264 – AB), a pharmaceutical company specialized in research, development and marketing of protein kinase inhibitors (PKIs), announces that the U.S. Food and Drug Administration (FDA) has granted the company Orphan Drug designation for masitinib in the treatment of esophagogastric adenocarcinoma.

The FDA's Office of Orphan Drug Products Development reviews applications for Orphan Drug status to support development of medicines for underserved patient populations, or rare disorders that affect fewer than 200,000 people in the United States. The successful application submitted by AB Science and the FDA granting of Orphan Drug status entitles the company to a seven-year period of marketing exclusivity in the United States for masitinib, if it is approved by the FDA for the treatment of esophagogastric adenocarcinoma. Orphan Drug status also enables the company to apply for research grant funding for phase I and II clinical trials, tax credits for certain research expenses, and a waiver from the FDA's application user fee, as well as additional support from FDA and a potentially faster regulatory process.

AB Science has recently communicated on the masitinib phase 2 results for this indication, which compare favorably with numerous published results for second-line irinotecan treatment.

In that prospective, open-label, randomized study, patients received masitinib in combination with irinotecan, or FOLFIRI (irinotecan, 5-fluorouracil and folinic acid), or 5-fluorouracil, after progression to platinum-based first-line chemotherapy. At the latest data cut-off (September 2015):

- In the masitinib plus irinotecan treatment-arm, median overall survival (OS) was 11.0, which compares favorably to the median OS benchmark for single-agent irinotecan, which has been reported at approximately 7.5 months (meta-analysis based on seven studies<sup>1</sup>). Safety data showed that the combination of masitinib and irinotecan had an acceptable safety profile.
- Of note, one patient treated with masitinib plus irinotecan showed a complete response, which is an exceptional observation in this clinical setting.
- In the masitinib plus FOLFIRI treatment-arm, median OS was 7.8. Again, safety data showed that the combination of masitinib and FOLFIRI had an acceptable safety profile.
- The masitinib plus 5-fluorouracil treatment-arm was closed early due to lack of efficacy on PFS.

Professor Aziz Zaanani, of Hôpital Européen Georges Pompidou, Paris, France, a leading investigator on this phase 1b/2 clinical trial, will present this updated phase 2 data in greater detail at the 2015 European Cancer Congress, on the 28th of September 2015.

Based on the efficacy data generated from this phase 2 study and the acceptable safety profile of masitinib, AB Science launched a confirmatory phase 3 trial evaluating masitinib at 6 mg/kg/day in combination with irinotecan in second-line. The study's primary endpoint is overall survival.

According to the GLOBOCAN 2012 estimate of worldwide cancer burden, stomach cancer is the fifth most commonly diagnosed cancer globally and the third leading cause of cancer death<sup>2</sup>. The most common type of stomach cancer is adenocarcinoma, accounting for at least 90% of all stomach cancers.

The incidence of stomach cancer is reported at approximately 100,000 patients in the USA and European Union, with a mortality rate of approximately 70,000 patients<sup>3</sup>. Approximately 90% of patients present with metastatic disease in either regional or distant sites and it is estimated that 60% of patients progressing after first-line treatment of metastatic cancer can receive a second-line of treatment. Based on these estimates, the number of eligible patients for second-line treatment of metastatic esophagogastric adenocarcinoma is 50,000 patients per annum in the USA and Europe.

<sup>1</sup> References from meta-analysis

[1] Thuss-Patience PC et al., *Eur J Cancer*. 2011 Oct, 47(15):2306-14.

[2] Kang JH et al., *J Clin Oncol*. 2012 May 1, 30(13):1513-8.

[3] Higuchi K et al., *Eur J Cancer*. 2014 May, 50(8):1437-45.

[4] Satoh T et al., *Gastric Cancer*. 2014 Sep 5.

[5] Roy AC et al., *Ann Oncol*. 2013 Jun, 24(6):1567-73.

[6] Kucukzeybek Y et al., *Asian Pac J Cancer Prev*. 2012, 13(6):2771-4.

[7] Oba M et al., *Oncol Lett*. 2011 Mar, 2(2):247-251.

<sup>2</sup> [http://globocan.iarc.fr/Pages/fact\\_sheets\\_cancer.aspx](http://globocan.iarc.fr/Pages/fact_sheets_cancer.aspx)

<sup>3</sup> [www.cancer.gov/cancertopics/pdq/treatment/gastric/HealthProfessional](http://www.cancer.gov/cancertopics/pdq/treatment/gastric/HealthProfessional); <http://eco.iarc.fr/eucan/>;

<http://seer.cancer.gov/statfacts/>

### **About Orphan Drug Designation**

The FDA Office of Orphan Products Development (OOPD) mission is to advance the evaluation and development of products (drugs, biologics, devices, or medical foods) that demonstrate promise for the diagnosis and/or treatment of rare diseases or conditions. In fulfilling that task, OOPD evaluates scientific and clinical data submissions from sponsors to identify and designate products as promising for rare diseases and to further advance scientific development of such promising medical products.

The approval of an orphan designation request does not alter the standard regulatory requirements and process for obtaining marketing approval for investigational use. Sponsors must establish safety and efficacy of a compound in the treatment of a disease through adequate and well-controlled studies. However, the FDA review process may be speedier for Orphan Drugs than those which do not receive Orphan Drug designation.

### **About masitinib**

Masitinib is a new orally administered tyrosine kinase inhibitor that targets mast cells and macrophages, important cells for immunity, through inhibiting a limited number of kinases. Based on its unique mechanism of action, masitinib can be developed in a large number of conditions in oncology, in inflammatory diseases, and in certain diseases of the central nervous system. In oncology due to its immunotherapy effect, masitinib can have an effect on survival, alone or in combination with chemotherapy. Through its activity on mast cells and consequently the inhibition of the activation of the inflammatory process, masitinib can have an effect on the symptoms associated with some inflammatory and central nervous system diseases and the degeneration of these diseases.

### **About AB Science**

Founded in 2001, AB Science is a pharmaceutical company specializing in the research, development and commercialization of protein kinase inhibitors (PKIs), a class of targeted proteins whose action are key in signaling pathways within cells. Our programs target only diseases with high unmet medical needs, often lethal with short term survival or rare or refractory to previous line of treatment in cancers, inflammatory diseases, and central nervous system diseases, both in humans and animal health.

AB Science has developed a proprietary portfolio of molecules and the Company's lead compound, masitinib, has already been registered for veterinary medicine in Europe and in the USA. The company is currently pursuing fourteen phase 3 studies in human medicine in first-line and second-line GIST, metastatic melanoma expressing JM mutation of c-Kit, multiple myeloma, metastatic colorectal cancer, metastatic prostate cancer, pancreatic cancer, T-cell lymphoma, mastocytosis, severe persistent asthma, rheumatoid arthritis, Alzheimer's disease, progressive forms of multiple sclerosis, and Amyotrophic Lateral Sclerosis. The company is headquartered in Paris, France, and listed on Euronext Paris (ticker: AB).

Further information is available on AB Science website: [www.ab-science.com](http://www.ab-science.com).

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markets, including trends of the economic conjuncture, the financial markets and the markets on which AB Science is present.

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