



Paris, 12 November 2014, 5.45 pm

**The Data and Safety Monitoring Board recommends  
the continuation of the phase 2 study with masitinib  
in advanced hepatocellular carcinoma based on safety and efficacy data**

**AB Science SA** (NYSE Euronext – FR0010557264 – AB), a pharmaceutical company specialized in research, development and marketing of protein kinase inhibitors (PKIs), announces that the external Data and Safety Monitoring Board (DSMB) has recommended the continuation of its phase 2 study of masitinib in advanced hepatocellular carcinoma based upon review of the latest safety and efficacy data. The DSMB was created as part of the Company's clinical study evaluating masitinib in the treatment of advanced hepatocellular carcinoma

The objective of this phase 2 study is to evaluate the safety and efficacy of masitinib in combination with etoposide, or masitinib in combination with irinotecan in patients with advanced hepatocellular carcinoma and who relapsed after a first line therapy with sorafenib. The study primary endpoint is overall survival.

There are three objectives: to determine if at least one combination has a trend of superiority on overall survival as compared to the latest benchmark in this indication, to determine which combination has the best benefit/risk if any, to determine the best dose of both masitinib and chemotherapies. Those three objectives are considered pre requisite to move into phase 3.

This recommendation from the DSMB is encouraging because it confirms that the benefit risk balance for masitinib is positive based on the data currently generated in this study.

Dr. Yann TOUCHEFEU (Service d'Hépatogastroentérologie, CHU Hôtel-Dieu, Nantes-France), principal investigator of the study indicated that "There is a high unmet medical need for patients in second line treatment of advanced hepatocellular carcinoma. The median overall survival is around 5 months with current therapies. For that reason, if masitinib confirmed an acceptable safety profile and showed a trend of increased overall survival as compared with this current benchmark, phase 3 would be warranted and masitinib could provide an option for second-line treatment of patients with advanced hepatocellular carcinoma".

There is a growing incidence of Hepatocellular carcinoma worldwide. The incidence was of 86,000 cases in the USA and Europe in 2008, and the mortality rate was of 78,640 cases . It is estimated that by 2020 the number of cases will reach 105,000 in these geographies<sup>1</sup>.

Around 40%<sup>2</sup> of patients have advanced Hepatocellular carcinoma (BCLC stage C). These patients bear a dismal prognosis and are eligible as first line treatment to Nexavar (sorafenib) a multi kinase inhibitor. With, sorafenib<sup>3</sup>, the median treatment time is around 5.5 months, and the median OS is 9.5 months in BCLC C patients.

There is currently no approved standard in second line of treatment after failure with sorafenib. Masitinib is therefore addressing a clear unmet medical need.

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<sup>1</sup> IARC. <<http://www-dep.iarc.fr/>>; 2011 [accessed 01.11.11].

<sup>2</sup> Journal of Hepatology 2012 vol. 56 j 908–943

<sup>3</sup> N Engl J Med 2008;359:378–390.

Around 60% of patients progressing after sorafenib usually can still take a second line of treatment. With this hypothesis the number of eligible patients for second line treatment of advanced hepatocellular carcinoma is estimated to be 25,000 per annum in Europe and USA by 2020.

#### **About Hepatocellular Carcinoma**

Liver cancer is the sixth most common cancer (749,000 new cases), the third cause of cancer related death (692,000 cases). Hepatocellular carcinoma represents more than 90% of primary liver cancers.

Resection may benefit certain patients, albeit mostly transiently. Many patients are not candidates given the advanced stage of their cancer at diagnosis. In these patients, local ablative therapies, including radiofrequency ablation, chemoembolization, and potentially novel chemotherapeutic agents, may extend life and provide palliation. Only a fraction of all patients have access to transplantation

#### **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 thirteen 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, 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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