



Paris, 30 September 2014, 5.45 pm

The Data and Safety Monitoring Board recommends continuation of phase 3 study of masitinib in mastocytosis 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 3 study of masitinib in mastocytosis based upon review of the latest safety and efficacy data. The DSMB was created as part of the Company's pivotal clinical study evaluating masitinib in the treatment of mastocytosis.

This new recommendation corroborates the previous DSMB recommendation to continue this phase 3 study based upon the favorable result of a futility analysis in November 2013. That analysis tested the possibility of masitinib to demonstrate superiority over placebo on the primary analysis as defined in the study protocol.

The objective of this phase 3 study is to compare the safety and efficacy of masitinib with that of placebo in adult patients having smoldering or indolent systemic mastocytosis with severe handicaps/symptoms at baseline.

The final analysis of trial data is still planned for 2015.

Pr. Olivier Hermine, President of the scientific committee of AB Science indicated that “Mastocytosis is particularly relevant for the clinical development of masitinib, which has been designed to selectively target mast cells. The underlying assumption for the development of masitinib in inflammatory and neurological disorders is that mast cells play a key role in the organization and activation of the inflammation, by participating directly in the tissue destruction but also in recruiting and activating the other immunity cells.

In mastocytosis, patients present with multiple symptoms related to mast cell activation and mediator release, such as gastro-intestinal disorders, flush, muscle and bone pain but also neurological disorders, such as depression or impairment of cognitive functions. The demonstration of masitinib efficacy in reducing severe symptoms associated with mastocytosis can establish a clear link between the symptoms and mast cell activation, and the clinical benefit of targeting mast cells”.

Mastocytosis is an orphan disease characterized by an abnormal proliferation of mast cells either in bone marrow only or in several tissues. Mastocytosis comes in two main forms: indolent and aggressive. Indolent mastocytosis can be either cutaneous or systemic. The prevalence of Indolent Systemic Mastocytosis (ISM) is estimated at between 1/40,000 and 1/20,000¹ of the general population. The symptoms and handicaps are severe in about one third of the patients, hence an estimated target population for masitinib ranging from 1/120,000 to 1/60,000 of the general population.

Since the prevalence of Indolent Systemic Mastocytosis is reputed to be comparable across countries, the target population for masitinib could reach an estimated 20,000 adult patients in the world annually.

There is currently no registered treatment in severe systemic mastocytosis.

¹ <http://www.orpha.net> (Indolent systemic mastocytosis)

Masitinib received orphan drug status designation in mastocytosis, both at EMA and FDA.

Previous studies with masitinib in mastocytosis

Two phase 2 studies have been conducted among a total of 46 patients, in two sub-populations of patients suffering from indolent mastocytosis with handicap: a first study in patients who do not carry the D816V mutation on the c-Kit gene, and another in patients who do carry such a mutation.

When compared with baseline data, masitinib decreased the flush frequency by 54%, decreased the pruritus score by 45%, improved the depression status by 40% and decreased the fatigue score by 52%. The two studies generated results consistent with each other despite the fact that the first one enrolled patients without the c-Kit 816 mutation and the second one with this mutation, suggesting that masitinib acts by inhibiting not only c-Kit but also Lyn, thereby blocking the release of the mediators by the mast cell.

In addition, long term follow-up data of the two phase 2 studies showed sustainability of responses generated by masitinib. Two-thirds of the patients decided to enroll in the study's extension phase; 61% patients were treated for more than 1 year and 25% were still under masitinib after 5 years.

About masitinib

Masitinib is a new orally administered tyrosine kinase inhibitor that targets mast cells, important cells for immunity, as well as a limited number of kinases that play key roles in various cancers. Owing to its novel 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. Through its activity of inhibiting specific kinases that are essential in some oncogenic processes, masitinib may have an effect on tumor regression, alone or in combination with chemotherapy. Through its activity on the mast cell and certain kinases essential to the activation of the inflammatory cells and fibrosing tissue remodeling, masitinib can have an effect on the symptoms associated with some inflammatory and central nervous system 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 new class of targeted molecules whose action is to modify signaling pathways within cells. Through these PKIs, the Company targets diseases with high unmet medical needs (cancer, inflammatory diseases, and central nervous system diseases), in both human and veterinary medicines.

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, and is 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.

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