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AB Science has submitted to EMA the marketing authorization application for Masitinib in the Treatment of Severe Systemic Mastocytosis

Date of review process starting 26 April 2016

AB Science SA (NYSE Euronext - FR0010557264 - AB), a pharmaceutical company specializing in the research, development and commercialization of protein kinase inhibitors (PKIs), announces the filing for registration to European Medicines Agency (EMA) for masitinib in the treatment of adult patient with severe systemic mastocytosis unresponsive to optimal symptomatic treatment, with date of review process starting on 26 April 2016. Masitinib is the first treatment to be evaluated in this indication. Top-line results from this phase 3 were previously announced on 30 November 2015.

Filing to EMA for the Marketing Authorization of masitinib in severe systemic mastocytosis was done on the basis of results from a phase III study showing that masitinib was superior to optimal symptomatic treatment on the primary efficacy analysis as well as secondary efficacy analyses.

This phase 3 randomized study compared masitinib plus optimal symptomatic treatment versus placebo plus optimal symptomatic treatment in adult patients with severe systemic mastocytosis, with or without D816V mutation of c-Kit. Study results showed that masitinib administered at 6 mg/kg/day was superior to the comparator, as measured by the cumulative 75% response rate until week 24 on the handicaps of pruritus or flushes or depression or fatigue (4H75% response). The 4H75% response was 18.7% for the masitinib treatment-arm versus 7.4% for the placebo treatment-arm ($p=0.0076$, Odd ratio=3.63) in the mITT population (primary analysis). According to protocol, the primary efficacy analysis was performed in the modified intent-to-treat population (mITT), yet the study was also successful on the sensitivity analysis performed in the intent-to-treat population (18.7% versus 7.6%, respectively, 0.0102, Odd ratio= 3.28).

Success in the primary analysis was also supported by positive outcomes in all secondary analyses.

Alain Moussy, CEO of AB Science, said *"The acceptance of this Marketing Authorization Application by EMA shows that authorities consider masitinib as a potential candidate for registration in this orphan disease for which there is currently no approved drug."*

EMA decision should be known during the first half of 2017.

About Mastocytosis

Mastocytosis is an orphan disease characterized by an abnormal proliferation or activation of mast cells either in the skin or in bone marrow or other organs. Mastocytosis comes in two main forms: indolent and aggressive. Indolent forms of mastocytosis can be either cutaneous or systemic. The prevalence of indolent systemic mastocytosis, including smoldering systemic mastocytosis, is estimated to be 1/26,000 in Europe¹. The symptoms and handicaps are severe in about one third of the patients; hence, an estimated target population for masitinib of approximately 1/78,000 of the general population.

Since the prevalence of indolent forms of systemic mastocytosis is reputed to be comparable across countries, the target population for masitinib could reach 10,000 adult patients in the USA and in Europe.

1: Prevalence of rare diseases: Bibliographic data, Orphanet Report Series, Rare Diseases collection, July 2015, Number 1: Listed in alphabetical order of disease or group of diseases.

http://www.orpha.net/orphacom/cahiers/docs/GB/Prevalence_of_rare_diseases_by_alphabetical_list.pdf

Orphan Drug Status

Masitinib has been granted orphan drug status in mastocytosis by both FDA and EMA.

There is currently no drug approved for the treatment of indolent mastocytosis.

Masitinib is the first drug to be evaluated in phase 3 in the indolent form of mastocytosis, systemic or not, severe or not.

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 microglia 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 twelve 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, severe asthma uncontrolled by oral corticosteroid, 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: <http://www.ab-science.com>

This document contains prospective information. No guarantee can be given as for the realization of these forecasts, which are subject to those risks described in documents deposited by the Company to the Authority of the financial markets, including trends of the economic conjuncture, the financial markets and the markets on which AB Science is present.

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