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AB Science announces that CHMP has adopted a negative opinion for masitinib in indolent systemic mastocytosis, primarily due to GCP inspection findings

AB Science has implemented the corrective actions to address the GCP findings

AB Science will ask for a re-examination with the opinion of a Scientific Advisory Group (SAG) to evaluate the benefit-risk balance

AB Science SA (NYSE Euronext – FR0010557264 – AB), a pharmaceutical company specialized in research, development and marketing of protein kinase inhibitors (PKIs), announces that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicine Agency (EMA) has adopted a negative opinion for masitinib in the treatment of adult patients with smouldering or indolent systemic severe mastocytosis unresponsive to optimal symptomatic treatments.

The objections precluding a recommendation of marketing authorisation by the CHMP pertained to the following principal deficiencies:

- The CHMP was concerned about the reliability of the study results because a routine GCP (good clinical practice) inspection at the study sites revealed serious failings in the way the study had been conducted.
- In addition, major changes were made to the study design while the study was ongoing, which made the results difficult to interpret.
- Finally, data on the safety of the medicine were limited and there were concerns regarding the medicine's side effects, including neutropenia (low levels of white blood cells) and harmful effects on the skin and liver, which were of relevance particularly because the medicine was to be used long term.

AB Science will ask for a re-examination based on the following grounds.

<u>GCP Findings</u>
The deficiencies concerning inspection findings have been corrected by AB Science and do not modify the study conclusions, both in terms of efficacy and safety assessment.
In the re-examination procedure, an independent audit of such corrective actions will be provided.

<u>Changes in the Study Design</u>
As it was detailed on the publication of the phase 3 results in *The Lancet*, protocol amendments were implemented between 3.5 years and 2 years prior to database unmasking, in order to increase benefit risk balance of the study. The main changes were the following:

1) Restrict the study to the patient population with greatest medical need, i.e. only patients with smouldering or indolent systemic mastocytosis with severe baseline symptoms of mast cell mediator release;
2) The threshold for positive treatment response was increased from 50% to 75%, thereby enhancing the clinical relevance of improvement;
3) Change in statistical methodology for the study primary analysis, from patient response at week 24 to overall response during week 8 to week 24 based on patient x handicap.

The first two changes were reviewed by the CHMP during a scientific advice procedure and were deemed acceptable and in principle desirable.

The third change was not discussed through scientific advice but was in line with EMA guideline on clinical trials in small populations (CHMP/EWP/83561/2005). In the re-examination procedure, AB Science will highlight that the original analysis on 75% patient response at week 24 remained positive considering the original sample size (25.8% % with masitinib versus 13.1% with placebo, p-value=0.0238), proving that this third modification did not modify the study conclusion.

Benefit-risk assessment and SAG

The size of the safety database is acceptable for orphan disease. In the re-examination procedure, AB Science will provide the CHMP with updated 2017 data from other studies, demonstrating that the long-term safety profile is acceptable.

The risk of masitinib has to be reassessed in light of the benefit, after correction of the GCP findings.

The benefit risk balance has been assessed as positive by many key opinion leaders of the scientific community based on results from this single pivotal study.

Professor Michel Arock, current Chair of the European Competence Network on Mastocytosis (ECNM), stated "*The risk-benefit balance of masitinib treatment in patients with smouldering or indolent severe systemic mastocytosis is positive based on the results of the phase 3 pivotal study. Masitinib is absolutely essential given the urgent medical need since the targeted patient population suffers from severe and recurrent symptoms, uncontrolled by optimal symptomatic treatments*."

A recent publication in *The Lancet* of the result of the phase 3 of masitinib in mastocytosis concluded that masitinib is an effective and well tolerated agent for the treatment of severely symptomatic indolent or smouldering systemic mastocytosis.

No Scientific Advisory Group (SAG) was convened during the initial assessment phase. In the re-examination procedure, AB Science will ask for SAG in order to provide an additional opinion on the assessment of the benefit-risk balance and on the urgency for a treatment for patients with smouldering or indolent systemic severe mastocytosis unresponsive to optimal symptomatic treatments.

Manufacturing

Of note, the CHMP did not raise any objection, major or minor, concerning the manufacturing of masitinib, meaning that all objections previously raised by the CHMP in its previous assessments were successfully resolved by AB Science.

Next step

The re-examination should lead the CHMP to deliver a second opinion in Q4 2017.

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 thirteen phase 3 studies in human medicine in metastatic prostate cancer, metastatic pancreatic cancer, relapsing metastatic colorectal cancer, relapsing metastatic ovarian cancer, GIST, metastatic melanoma expressing JM mutation of c-Kit, relapsing T-cell lymphoma, severe asthma, amyotrophic lateral sclerosis, Alzheimer's disease and progressive forms of multiple sclerosis. The company is headquartered in Paris, France, and listed on Euronext Paris (ticker: AB).

Further information is available on AB Science's website: www.ab-science.com.

Forward-looking Statements - AB Science
This press release contains forward-looking statements. These statements are not historical facts. These statements include projections and estimates as well as the assumptions on which they are based, statements based on projects, objectives, intentions and expectations regarding financial results, events, operations, future services, product development and their potential or future performance.

These forward-looking statements can often be identified by the words "expect", "anticipate", "believe", "intend", "estimate" or "plan" as well as other similar terms. While AB Science believes these forward-looking statements are reasonable, investors are cautioned that these forward-looking statements are subject to numerous risks and uncertainties that are difficult to predict and generally beyond the control of AB Science and which may imply that results and actual events significantly differ from those expressed, induced or anticipated in the forward-looking information and statements. These risks and uncertainties include the uncertainties related to product development of the Company which may not be successful or to the marketing authorizations granted by competent authorities or, more generally, any factors that may affect marketing capacity of the products developed by AB Science, as well as those developed or identified in the public documents filed by AB Science with the Autorité des Marchés Financiers (AMF), including those listed in the Chapter 4 "Risk Factors" of AB Science reference document filed with the AMF on November 22, 2016, under the number R. 16-078. AB Science disclaims any obligation or undertaking to update the forward-looking information and statements, subject to the applicable regulations, in particular articles 223-1 et seq. of the AMF General Regulations.

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