Paris, June 22, 2017, 5.45pm

AB Science Presents Supportive Data from its Phase 3 Study in Severe Systemic Mastocytosis at the 22nd Congress of the European Hematology Association (EHA)

Abstract Selected for Presentation by the EHA Scientific Program Committee

AB Science SA (NYSE Euronext - FR0010557264 - AB), a pharmaceutical company specializing in the research, development and commercialization of protein kinase inhibitors (PKIs), today announced that supportive data analyses from its phase 3 trial in severe systemic mastocytosis will be presented as a poster presentation at the International 22nd Congress of the European Hematology Association (June 22 - 25, 2017, Madrid, Spain).

Professor Olivier Hermine, President of the Scientific Committee of AB Science and coordinator of the Reference Center for Mastocytosis (CeReMast, Paris, France), will deliver this presentation at the EHA 2017 Congress on Saturday, 24th June. Abstracts were selected by the EHA Scientific Program Committee based on scientific quality, with just one-fifth of submitted abstracts being selected for poster presentation at the meeting. The masitinib international AB06006 study was the first ever phase 3 prospective, randomized placebo-controlled study of a treatment for severe indolent systemic mastocytosis in patients who are unresponsive to existing, optimal symptomatic treatment. There is currently a very high unmet medical need in this population.

Following inspection report conclusions performed as part of the procedure for the EMA marketing authorization of masitinib in the treatment of mastocytosis, in which GCP deviations have been observed in the conduct of the study, AB Science implemented the required corrective and preventive actions and performed a reassessment of the study results, within this new quality system. These actions do not modify the study results.

- Regarding efficacy data, sensitivity analyses performed by excluding some data with quality below standard and related to depression score do not modify the study results
- Regarding safety data, a full remonitoring was performed, followed by a medical reassessment of all adverse events. These actions do not modify the masitinib safety profile

"The main study results, published earlier this year by The Lancet[1], reported a significant and clinically meaningful treatment benefit for masitinib versus placebo" said Professor Michel Arock, current Chair of the European Competence Network on Mastocytosis (ECNM). *"Here additional analyses are presented, that aide interpretation of the study's predefined primary endpoint using more conventional measures of patient response. These data confirm the clinical relevance, durability, and generalizability of the positive primary endpoint from study AB06006. Taken together with the main efficacy and safety data, these supportive analyses reinforce the positive benefit/risk balance for masitinib in this difficult-to-treat population."*

Key data are shown below:

- Masitinib showed a significant improvement over placebo according to its pre-specified primary endpoint, with a cumulative response of 18.7% versus 7.4%, respectively, P=0.008.
- This primary endpoint was based on repeated measures methodology for rare diseases via the generalized estimating equation (GEE) model, an established technique that makes a longitudinal analysis with respect to symptoms as opposed to the more conventional patient response rate at a single time-point.
- Computing treatment-effect according to cumulative response per patient (GEE model) confirmed this positive outcome on the primary endpoint: 26.7% versus 12.8%, respectively, P=0.0212.
- Computing treatment-effect according to individual patient response (Pearson chi-square) was also significant for masitinib: 40.3% versus 24.2%, respectively, P=0.0062.

- Response (per patient) on all severe baseline symptoms for at least one visit was: 16.4% for masitinib versus 1.6% for placebo, P=0.0062.

Finally, analysis of sustained response in all severe baseline symptoms over multiple visits was highly discriminatory between treatment-arms:

- For patients with 3 severe baseline symptoms masitinib generated a 12.5% response rate (≥75% improvement in each symptom) for 3 out of 5 visits, versus no response for placebo.
- For patients with 2 severe baseline symptoms masitinib generated a response rate of 21.1%, 15.8% and 10.5% over at least 1, 2, and 3 visits, respectively, versus no response for placebo.

[1] Lortholary et al. Lancet. 2017 Feb 11;389(10069):612-620

➢ **Abstract and schedule**

MASITINIB FOR TREATMENT OF SEVERELY SYMPTOMATIC INDOLENT SYSTEMIC MASTOCYTOSIS: ADDITIONAL EFFICACY ANALYSES FROM THE RANDOMIZED, PLACEBO-CONTROLLED, PHASE 3 STUDY
Abstract: #P709
Session Title: Other Non-malignant hematopoietic disorders
Date, Location: Saturday, June 24 (17:30), Hall 7

This abstract will be published in the congress abstract book (a special addition of the Haematologica journal), available both at http://www.haematologica.org and https://www.ehaweb.org.

➢ **About the phase 3 study in severe systemic mastocytosis**

The phase III study results showed 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, Odds ratio=3.63) in the mITT population (primary analysis). Success in the primary analysis was also supported by positive outcomes in secondary analyses.

➢ **Targeted population with masitinib in 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 Europe[2]. 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.

[2] 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 the 22nd Congress of the European Hematology Association (June, 2017, Madrid, Spain)
The EHA Annual Congress is the premier hematology congress in Europe providing a forum for presenting original unpublished data and sharing ideas for hematological innovation as well as disseminating evidence-based knowledge of primary clinical relevance. Located in a different European city each year, this meeting attracts more than 10,000 professionals with an interest in hematology from around the world, with a program which aims to promote excellence in clinical practice, hematological research and education.
https://www.ehaweb.org/congress-and-events/22nd-congress/key-information/

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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