



Paris, 30th of May 2016, 8am

AB Science Will Present Phase 3 Data in Severe Systemic Mastocytosis at the International 21st Congress of the European Hematology Association (EHA)

Abstract Selected for Oral 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 results from its phase 3 trial in severe systemic mastocytosis will be presented as an oral presentation at the 21st Congress of the European Hematology Association (June 9 - 12, 2016, Copenhagen, Denmark).

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 oral presentation at the EHA 2016 Congress on Sunday, 12th June. Abstracts were selected by the EHA Scientific Program Committee based on scientific quality, with just 8% of the total submitted being selected for oral presentation.

Professor Olivier Hermine said: *"We will present results from the masitinib international AB06006 study, which was the first ever phase 3 prospective, randomized, placebo-controlled study of a treatment for severe indolent systemic mastocytosis, including the subvariant of smouldering systemic mastocytosis. There is currently no registered or established standard treatment for this rare condition with high unmet medical need. Study AB06006 successfully achieved all of its primary and secondary objectives. The study also generated positive results on the pre-specified objective markers of mast cell activation. Masitinib may therefore be an important new treatment option for these patients; moreover, both efficacy and safety data indicate a possibility for effective long-term management of this difficult-to-treat condition."*

➤ **Abstract and schedule**

Masitinib for the treatment of severely symptomatic indolent and smouldering systemic mastocytosis: a randomized, placebo-controlled, international, phase 3 study

Abstract: #S828

Session Title: Non-malignant hematopoietic disorders

Date, Location: Sunday, June 12 (8am), Room H5

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

The phase 3 study was designed to evaluate masitinib efficacy and safety in severe systemic mastocytosis patients, with or without D816V mutation of c-Kit. The primary objective of the phase 3 study was to detect a statistically significant difference between masitinib (plus optimal concomitant symptomatic treatments) and placebo (plus optimal concomitant symptomatic treatments) in cumulative response on four severe symptoms, referred to also as handicaps (pruritus, flush, depression and asthenia).

➤ **Targeted population with masitinib in severe systemic 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 the 21st Congress of the European Hematology Association (June, 2016, Copenhagen, Denmark)

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.

<http://www.ehaweb.org/congress-and-events/21st-congress/program/program-at-a-glance-2/>

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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AB Science – Financial Communication & Media Relations

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