Paris, 06 October 2015, 6pm

Simulations based on pooled phase 2 study data in severe systemic mastocytosis for future comparison with phase 3 population will be presented this month at two international scientific conferences

Unblinding of phase 3 data planned for November 2015

AB Science SA (NYSE Euronext - FR0010557264 - AB), a pharmaceutical company specializing in the research, development and commercialization of protein kinase inhibitors (PKIs), today announced the communication of new phase 2 data in systemic severe mastocytosis aimed at simulating the targeted phase 3 study population and response criteria. Based on pooled data taken from the two phase 2 studies in mastocytosis, results are indicative of the outcome expected for the on-going phase 3 study.

Findings from this simulation are to be presented at the next congress of the European Competence Network on Mastocytosis (ECNM) (October 8-10 2015 in Munich, Germany) and also at the International European Mast Cell and Basophil Research Network (EMBRN) meeting (October 21-23 2015 in Marseille, France).

Definition of primary endpoint for phase 3 in severe systemic mastocytosis

AB Science is near to complete the first phase 3 clinical trial ever in mastocytosis. The design is to evaluate masitinib efficacy in severe systemic mastocytosis patients, with or without D816V mutation of c-Kit. The objective (primary endpoint) of the phase 3 study is to detect a statistically significant difference between masitinib (plus concomitant symptomatic treatments) and placebo (plus concomitant symptomatic treatments) in cumulative response on four severe symptoms, named handicaps.
Patients enrolled in the phase 3 study can have between one and four of the following severe mastocytosis-related symptoms at baseline:

- Pruritus score ≥ 9
- Number of flushes per week ≥ 8
- Depression measured by Hamilton rating scale (HAMD-17) score ≥ 19
- Asthenia measured by Fatigue Impact Scale total score ≥ 75

At each patient evaluation between weeks 8 and 24, each of the above severe symptoms is evaluated. An improvement ≥ 75% with respect to baseline in one symptom is recorded as one positive treatment response.
The primary analysis is based on the comparison between masitinib and placebo in the number of actual responses between week 8 and week 24 divided by the total number of possible responses over the same treatment period.
This endpoint is referred to below as the "4 Handicaps 75% Response or 4H75%-response".

Pooled phase 2 studies simulation of phase 3 population and response criteria

Prior to conducting this phase 3 trial, AB Science completed two phase 2 studies, one study included systemic mastocytosis patients expressing the D816V mutation of c-Kit, and another study included systemic mastocytosis patients without the D816V mutation of c-Kit in at least one organ.
These two studies comprised a total of 46 patients, including 28 diagnosed with a severe systemic mastocytosis which consists of the population of the phase 3 efficacy analysis.

Key results from these pooled phase 2 data are as follows:

- The overall cumulative 75% response rate (4 Handicaps 75% Response) was 26.1% in the pooled population of systemic patients with severe symptoms at baseline. The 75% response rate (non cumulative) at week 24 was 23.5% in the same population.

- 52.8% and 28.6% of patients suffering respectively from the mastocytosis-related symptoms of severe flush and severe pruritus, responded to masitinib treatment (based on the cumulative 75% response rate), with a reduction of the symptom by at least 75% with respect to baseline.

- There was no significant difference in response to masitinib between mastocytosis patients with wild type c-Kit or D816V mutation of c-Kit.

- 78% of the overall pooled mastocytosis population completed the protocol period and participated to the extension phase after week 24. Furthermore, 24% of patients were still receiving masitinib treatment after 6 years, suggesting a lifetime benefit with masitinib may be possible.

Placebo effect and expected difference with masitinib

The following conclusions can be drawn from this new data analysis aimed at simulating the targeted phase 3 study population and response criteria:

a) For phase 3 to confirm phase 2, the expected 4 Handicaps 75% Response is expected to be around 20%.
b) Placebo effect is unknown since there has been no previous controlled study in indolent mastocytosis.
c) The phase 3 objective is to detect a statistical difference in the 4H75%-response criterion between masitinib and placebo with p-value < 0.05. A 10% difference would reach this objective.
d) A 4H75%-response of around 20% for the masitinib treatment-arm and a difference with placebo of around 10% is widely interpreted as representing a relevant clinical benefit, as evidenced by comparison with analogous response criterion reported for a recently registered drug in rheumatoid arthritis.
    - The 4H75%-response criterion used in the phase 3 mastocytosis study signifies an almost complete disappearance of symptoms
    - Rheumatoid arthritis studies use ACR20, ACR50, and ACR70 criteria, which measure respectively a reduction by 20%, 50%, and 70% of disease symptoms. ACR70 is a comparable endpoint to the 4H75%-response criterion
    - The latest drug registered in rheumatoid arthritis generated[1] an ACR70 ranging from 13.2 to 19.9 (mean 15.3) versus placebo ranging from 1.3 to 5.8 (mean 2.7)

Comment from expert in mastocystosis

Olivier Hermine is President of the scientific committee of AB Science and coordinator of the Reference Center for Mastocytosis in France (CeReMast), which is one of the largest centers worldwide in terms of mastocytosis patient's population. He commented these findings: *"A 75% reduction in severe symptoms at baseline is a relevant clinical benefit. The major response observed on flush and pruritus is interesting because it proves that masitinib has a strong biological activity against the activation of mast cells as these two symptoms are well recognized to be associated with mast cell activation in mastocytosis. Also, the long-term follow-up data suggest that the observed treatment effect is maintained over time".*

[1] http://www.fda.gov/downloads/AdvisoryCommittees/CommitteesMeetingMaterials/Drugs/ArthritisAdvisoryCommittee/UCM304200.pdf

Phase 3 status

The phase 3 is currently closed to enrolment. The unblinding of phase 3 results is planned for November 2015 once all data have been collected and the database cleaned. Communication of results will be done soon after.

Targeted population with masitinib in mastocytosis

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 forms of mastocytosis can be either cutaneous or systemic. The prevalence of indolent mastocytosis is estimated at between 1/20,000 and 1/10,000 of the general population. Since systemic form of mastocytosis is estimated to account for 50% of indolent mastocytosis, the prevalence of indolent systemic mastocystosis (ISM) is estimate at between 1/40,000 and 1/20,000[2] 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 systemic forms of indolent 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.

Orphan Drug Status

Masitinib was granted orphan drug status in mastocytosis by both FDA and EMA.
There is currently no drug approved for the treatment of 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 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 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, mastocytosis, severe persistent asthma, 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.

[2] http://www.orpha.net (Indolent systemic mastocytosis)

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