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AB Science announces successful non futility test for masitinib in progressive forms of multiple sclerosis

Independent Data Safety Monitoring Committee recommends continuation of phase 3 study based on review of safety and efficacy data

Company to host web conference on masitinib in multiple sclerosis on 28 July 2015, from 5.35pm to 6.35pm CET

AB Science SA (NYSE Euronext – FR0010557264 – AB), a pharmaceutical company specialized in research, development and marketing of protein kinase inhibitors (PKIs), today announced the successful completion of a futility analysis related to the masitinib phase 3 trial for the treatment of patients with primary progressive or relapse-free secondary progressive multiple sclerosis. Based on these results, the Independent Data Safety Monitoring Committee (IDMC) has recommended the continuation of the study.

Phase 3 status

The ongoing phase 3 trial is a double-blind, randomized, placebo-controlled study (AB07002) designed to assess the safety and efficacy of masitinib in patients with primary progressive or relapse-free secondary progressive multiple sclerosis. The treatment period is 96 weeks.
The main measures to assess efficacy of masitinib in this disease are:

- Change in MSFC (Multiple Sclerosis Functional Composite), which is a three-part assessment instrument to measure leg function/ambulation, arm/hand function, and cognitive function.
- Change in the MSQOL-54 (Multiple Sclerosis Quality of Life 54 items), which is a quality of life measure.
- Change in EDSS (Expanded Disability Status Scale), which is a scale used for quantifying disability in multiple sclerosis and monitoring changes in the level of disability over time.

The study is intended to enroll 600 patients.

This study was assessed as non-futile by the IDMC. The characteristics of the futility test were as follows:

- Performed after approximately one third of the planned study population had reached the 48 week treatment duration period
- Hypothesis that all the remaining patients to be enrolled in the study will follow the trend observed on the patient already enrolled at the time of futility analysis.
- P-value below 5%
- Conditional power (predictive probability of success) of 20%

More importantly, the IDMC highlighted that the study was not futile on the three main assessment criteria, MSFC, MSQOL-54, and EDSS.

A futility analysis tests the inability of a clinical study to achieve its efficacy objective. Therefore, a conclusion that a study is not futile suggests that a clinical study has the potential to achieve its stated efficacy objective.

The study previously successfully passed all safety data reviews by the IDMC, indicating that there is no major or unexpected safety concern with masitinib in this patient population.

"*This positive outcome of the futility test is good news because there is no registered treatment in this indication*" said Professor Ramió-Torrentà of Dr Josep Trueta University Hospital, Girona, Spain and one of the leading investigators on this study. "*All drugs registered in relapsing forms of multiple sclerosis, recently or not, have failed so far to demonstrate efficacy in the progressive forms of multiple sclerosis. Furthermore, masitinib is not an immunosuppressive drug, unlike drugs used in relapsing forms of multiple sclerosis.*"

Alain Moussy, CEO and co-founder of AB Science commented "*We are very pleased with this outcome as our three on-going phase 3 studies in neurology, in progressive forms of multiple sclerosis, in Alzheimer's disease, and in amyotrophic lateral sclerosis, are all non futile. This was not obvious since those three indications have remained so far a challenge for the pharmaceutical industry. This may be indicative of the potential of masitinib for the treatment of neurodegenerative disorders*."

The next step for this study is a second futility analysis once one third of patients have reached the 96 week time point. Then an interim analysis is expected once 50% of patients planned to be enrolled have reached the 96 week treatment duration period.

Web conference

AB Science will be hosting in the next few days a web conference focused on masitinib for the treatment of multiple sclerosis. This event will feature key opinion leaders in the field of multiple sclerosis treatment.

The web conference call will be held on 28 July 2015 from 5.35pm to 6.35pm CET.

The web conference will provide the opportunity to understand:

- The current positioning of masitinib in MS
- The scientific rationale for developing masitinib in MS
- The efficacy and safety update on the on-going phase 3 study
- The market potential for masitinib in MS

It will be followed by a Q&A session.

The following experts in this disease will animate the discussion and answer questions.

- Dr Angela Genge
  Montreal Neurological Institute and Hospital
  Montreal, Canada

- Lluís Ramió i Torrentà, MD, PhD
  Department of Neuroimmunology and multiple sclerosis University Hospital of Girona
  Girona, Spain

- Pr Olivier Hermine
  Division of Hematology/Oncology, Necker University Hospital
  Paris, France

The presentation supporting this conference call will be made available on AB science web site upon completion of the web call.

Scientific rationale

Multiple sclerosis (MS) is an inflammatory condition that damages the myelin of the central nervous system, leading to neurologic impairment and possibly severe disability. MS is characterized by chronic patchy inflammation of the central nervous system with demyelination.

Masitinib is a selective tyrosine kinase inhibitor that is particularly efficient in controlling the survival, migration and degranulation of mast cells (and thus indirectly controlling the array of proinflammatory and vasoactive mediators these cells can release), through inhibition of essential growth and activation signaling pathways.

Several findings support the hypothesis that mast cells, which are found on both sides of the blood–brain barrier (BBB), actively participate in the pathogenesis of MS and also experimental allergic encephalomyelitis (EAE), an animal model of human demyelinating diseases. To this end, the ability and effect of masitinib in the inhibition of mast cell function in MS was explored using an EAE murine model considered to be a model for all progressive forms of MS. Treatment of mice with masitinib led to a significant reduction in disease relative to control mice. A masitinib dose-dependent effect was also evident. Thus, molecules, like masitinib, able to inhibit the survival and/or activation of mast cells may be able to control the symptoms and progression of MS or any related disease.

As a reminder, proof of concept for the evaluation of masitinib in progressive forms of multiple sclerosis was established through a double-blind, placebo-controlled phase 2 study with thirty-five patients. This study showed a trend of efficacy with masitinib in improving relevant function (leg/ambulatory, arm/hand and cognitive functions) of patients suffering from both PP-MS and SP-MS as measured by MFSC (Multiple Functional Sclerosis Composite). The phase 2 results were published in BMC Neurol. 2012 Jun 12;12:36. doi: 10.1186/1471-2377-12-36.

Targeted population

Four principal courses of MS are currently defined according to clinical characteristics; namely: Relapsing Remitting MS (RRMS), Secondary Progressive MS (SPMS), Primary Progressive MS (PPMS), and Progressive Relapsing MS (PRMS). The disease typically presents as RRMS, with more than 50% of RRMS patients entering a progressive phase (SPMS) following a highly variable delay. Approximately 10 to 15% of patients present with PPMS, which is characterized by continuous disease progression from the onset of disease, i.e. without relapses and remissions, for which prognosis is considered as poor due to the relatively rapid development of advanced disability as compared with RRMS.

Altogether, the progressive forms of multiple sclerosis represent around 60% of patients, hence around 400,000 patients in the USA and in the EU alone.

In general, drugs used in the treatment of MS are considered to act as immunomodulators, with the aim to decrease relapse rate, modify relapses, and diminish the accumulation of disability over time. Despite these approved therapies, many of which require parenteral administration, the unmet medical need in MS treatment remains substantial, especially for the subpopulations of PPMS and relapse-free SPMS (rfSPMS) for which there are currently no treatments proven to slow disease progression.

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 fourteen 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, rheumatoid arthritis, 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: .

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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