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AB Science announces positive top-line Phase 3 results for oral masitinib in severe asthma

This is the second Phase 3 study to demonstrate efficacy for masitinib in severe asthma

AB Science SA (Euronext - FR0010557264 - AB) today announced that the Phase 3 study (AB14001) evaluating oral masitinib in severe asthma uncontrolled by high-dose inhaled corticosteroids (ICS) and with eosinophil level >150 cells/µL met its primary endpoint.

The pre-specified primary analysis was rate of severe asthma exacerbations, with masitinib demonstrating a statistically significant 29% reduction in severe exacerbations relative to placebo (p=0.022). The frequency of severe asthma exacerbations was 0.43 in the masitinib arm, versus 0.62 in the placebo arm. Duration of exposure was well-balanced between the treatment-arms (16 months in the masitinib arm and 17 months in the placebo arm). Sensitivity analysis based on the rate of moderate and severe asthma exacerbations was consistent with the primary analysis and detected a statistically significant 31% reduction in exacerbations (p=0.005) between masitinib and placebo. The frequency of moderate and severe asthma exacerbations was 0.55 in the masitinib arm, versus 0.80 in the placebo arm.

This is the second time that masitinib has demonstrated efficacy in reducing severe asthma exacerbations in patients with severe asthma. The treatment effect observed in study AB14001 is comparable with the effect previously reported for study AB07015. In that first phase 3 study, which evaluated masitinib in severe asthma uncontrolled by oral corticosteroids (OCS), masitinib significantly (p=0.010) reduced the rate of severe asthma exacerbations by 35% as compared with placebo. The frequency of severe asthma exacerbations in study AB07015 was 0.34 in the masitinib arm, versus 0.45 in the placebo arm. Duration of exposure was also well-balanced between the treatment arms (13 months in both treatment arms).
Safety was consistent with the known tolerability profile for masitinib.

Detailed results will be presented at an upcoming medical meeting.

The study AB14001 enrolled patients with blood eosinophil level >150 cells/µL, which differs from the population usually addressed by biological treatments, targeting patients with high eosinophils (>300 cells/µL or above) defined as Th2-high eosinophilic asthma.

Masitinib is a *first in class* drug in severe asthma, distinct from biological treatments targeting type-2 high eosinophilic phenotypes of asthma. Masitinib has a dual mechanism of action, targeting mast cells and PDGFR signaling that are both involved in airway remodeling associated with severe asthma. It has also been shown that increased mast cell activity is associated with both eosinophilic (Th2-high) and non-eosinophilic (Th2-low) asthma phenotypes. Furthermore, masitinib is orally administered, whereas biologics are sub-cutaneous, which is an advantage because oral administration is less of a burden for patients and facilitates compliance for long-term use.

There is still a need for effective therapy of patients with severe asthma. Biologics are established in first line treatment in severe asthma patients with blood eosinophil levels of ≥300 cells/µL. However, these therapies have limited efficacy in reducing severe asthma exacerbations for severe asthmatics with blood eosinophil levels of <300 cells/µL. In addition, an estimated 33% to 60% of severe eosinophilic (Th2-high) asthma patients have sub-optimal response or are in failure to type 2 targeted therapeutics.

Asthma uncontrolled by high dose inhaled corticosteroid is estimated at 1,500,000 people[1,2] in the USA and in the EU. Among these patients, it is estimated that 75% (i.e. 1,125,000) have blood eosinophil levels of ≥150 cells/µL.

"We are very pleased that this study demonstrated efficacy of masitinib in severe asthma uncontrolled by high dose inhaled corticosteroids. After the first positive results of masitinib in severe asthma uncontrolled by oral corticosteroids, this study confirms the efficacy of masitinib in severe asthma population. Taken together, we now have two pieces of evidence that masitinib is effective in severe asthma with an eosinophil level above 150 cells/µL, which represents a broader population that the one usually addressed by biologic therapies. These two results seem sufficiently robust to claim that masitinib is a serious candidate as a new oral treatment option for severe asthma", said Lavinia Davidescu, MD, PhD, principal coordinating investigator of the study.

"*This is indeed the second positive large-scale study with masitinib in patients with severe asthma not restricted to Th2-high asthma phenotypes, which represents an unmet medical need population. In addition, it is important to highlight that masitinib offers a totally new mechanism of action as compared with available treatment options in asthma",* said Pascal Chanez, Professor of Respiratory Diseases at Aix-Marseille University, France.

Intellectual Property for masitinib is secured in severe asthma until 2032. The U.S. Patent and Trademark Office has granted a patent (13/983626) relating to methods of treating severe persistent asthma with masitinib. This patent, protects the use of masitinib in the treatment of severe persistent corticosteroid-dependent asthma and severe persistent corticosteroid-resistant asthma.

Phase 3 studies in asthma

Study AB14001 was a prospective, multicenter, randomized, double-blind, placebo-controlled, 2-parallel groups, phase 3 study evaluating the efficacy and safety of masitinib in asthma uncontrolled by high-dose inhaled corticosteroids and with eosinophil level (>150 cells/µL.

Eligible patients were patients with eosinophil level related to asthma at baseline ≥ 0.15 K/uL and with a physician diagnosis of persistent asthma for at least 12 months based on GINA 2009 Guidelines whose asthma is partially controlled or uncontrolled on ICS/LABA combination therapy based on the following criteria:

- A well-documented requirement for regular treatment in the 12 months prior to screening with or without maintenance oral corticosteroids (OCS)
- Forced expiratory volume (FEV1) ≥ 35 to < 80% predicted normal
- Reversibility of at least 12% and 200 ml in FEV1 after 200 µg to 400 µg or documented history of a reversibility test that met these criteria within 12 months prior to screening, or documented history of bronchial hyperreactivity from a positive methacholine challenge (PD20 methacholine ≤ 8 mg) within 12 months prior to screening
- Within the 12 months prior to screening at least 2 severe asthma exacerbations. Severe asthma exacerbation is defined by any of the following events:
    - Treatment with 1 or more systemic (oral and/or parenteral) steroid bursts for worsening asthma
    - In-patient hospitalization or an emergency care visit for worsening asthma

Participants received masitinib (3.0 mg/kg/day), given orally twice daily, with a dose escalation to 4.5 mg/kg/day after 4 weeks of treatment, followed by dose escalation to 6.0 mg/kg/day after 4 weeks of treatment. Each ascending dose titration was subjected to a safety control.

The primary endpoint of this study was the annualized severe asthma exacerbation rate for the overall time on treatment, as for study AB07015 in severe asthma uncontrolled with OCS.

References

1. Respir Med. 2006 Jul;100(7):1139-51. Epub 2006 May 18.
   Prevalence ranges from 7% (France, Germany) to 11% (USA) and 18% (UK). Average 10%. Rising incidence
2. J Investig Allergol Clin Immunol 2012; Vol. 22(7): 460-475
   20% of asthma patients have asthma requiring high dose inhaled or oral corticosteroids
   20% of these asthma patients are uncontrolled.
   Only 55% of patients initially suspected of having asthma uncontrolled by high dose ICS or OCS receive a confirmed diagnosis

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.

AB Science has developed a proprietary portfolio of molecules and the Company's lead compound, masitinib, has already been registered for veterinary medicine and is developed in human medicine in oncology, neurological diseases, and inflammatory diseases. 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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