



Paris, 13 January 2015, 5.40pm

## **The Data and Safety Monitoring Board recommends the continuation of phase 3 study of masitinib in amyotrophic lateral sclerosis based on safety data**

**AB Science SA** (NYSE Euronext – FR0010557264 – AB), a pharmaceutical company specialized in research, development and marketing of protein kinase inhibitors (PKIs), announces that the external Data and Safety Monitoring Board (DSMB) has recommended the continuation of its phase 3 study of masitinib in amyotrophic lateral sclerosis (ALS) based upon review of the latest safety data. The DSMB was created as part of the Company's pivotal clinical study evaluating masitinib in the treatment of amyotrophic lateral sclerosis.

The on-going phase 3 clinical trial is an international, multicenter, randomized, double-blind study comparing the efficacy and safety of masitinib with that of placebo in the treatment of patients with amyotrophic lateral sclerosis. Study treatment is given as add-on therapy to patients who have been treated with a stable dose of riluzole. The efficacy analysis is measured by the Amyotrophic Lateral Sclerosis Functional Rating Scale (ALSFRS-Revised) after 48 weeks of treatment.

These results are reassuring because they confirm there are no observed safety concerns with masitinib in combination with riluzole over a 1-year treatment period.

Professor Olivier Hermine, president of the scientific committee of AB Science explained: *"In this study, we assume that mast cells, which are key immune cells, actively participate to the pathogenesis of ALS, through the release of mediators that sustain the inflammatory network of the central nervous system. Mast cells, which are present in large quantities in the brain and in the spinal cord, could also influence the survival and function of motor neurons, and thus participate to the pathophysiology of ALS. Since masitinib is a selective inhibitor of c-Kit and Lyn, two kinases that play a major role in the survival and activation of mast cells, it may lead to positive effects on the symptoms of the pathology."*

Dr. Luis Barbeito (Institut Pasteur de Montevideo - Uruguay) commented: *"We completed several studies in animal models suggesting that masitinib might offer therapeutic benefits in ALS patients alone or in combination with riluzole. Notably, masitinib demonstrated in transgenic ALS murine model a delay in the onset of symptoms, improvement in grip strength, reduction in weight loss and a trend of increased survival. Masitinib also prevented the increase in tryptase expression - a marker of inflammation, and perivascular mast cell number, and prevented motoneuron pathology and death in a murine model for ALS, thus suggesting the implication of mast cells. We are currently conducting additional animal experiments to better elucidate the precise mechanism of action of masitinib in this disease, yet we believe the body of evidence we already have strongly supports the on-going phase 3 study in ALS."*

Amyotrophic lateral sclerosis is a rare degenerative disorder that results in progressive wasting and paralysis of voluntary muscles. There are approximately 30,000 people with ALS in the European Union and 15,000 in the US, with more than 7,500 new cases diagnosed each year in Europe and 4,500 in the US. Almost 50% of ALS patients die within 3 years and 90% die within 5 years.

### **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, 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: [www.ab-science.com](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.*

\* \* \*

AB Science – Financial Communication & Media Relations  
[investors@ab-science.com](mailto:investors@ab-science.com)