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New preclinical data demonstrate that masitinib has an unexpected protective effect on muscles and nerves in amyotrophic lateral sclerosis (ALS)

AB Science SA (NYSE Euronext – FRO010557264 – AB), a pharmaceutical company specialized in the research, development and marketing of protein kinase inhibitors (PKIs), announces today that new mechanistic data demonstrate the unexpected protective effect of masitinib on muscles and nerves in amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease. These new data have been presented at the 27th International Symposium on ALS/MND in December 2016 in Dublin, Ireland.

These new data complete the following previous findings:

- The preclinical data demonstrating the inhibiting effect of masitinib on microglial cells and survival prolongation in post-paralytic SOD1^{G93A} rats
- The clinical data from the phase 2/3 interim analysis showing the positive effect of masitinib on the functional score ALSFRS-R, which is a rating instrument for monitoring the progression of disability in patients with ALS and which correlates significantly with quality-of-life and survival.

Professor Luis Barbeito (Head of the Neurodegeneration Laboratory, Institut Pasteur in Montevideo, Uruguay), delivered a presentation at the 27th International Symposium on ALS/MND (December 2016, Dublin, Ireland). This meeting is the largest medical and scientific conference specific to ALS and is the premier event in the ALS research calendar for discussion on the latest advances in research and clinical management.

Professor Luis Barbeito said *"Beyond our recently published findings¹, we have acquired additional preclinical data showing neuroprotective effects of masitinib in ALS. We have now shown that masitinib generates its observed neuroprotective effect in ALS by regulating neuroinflammation in the peripheral nervous system as well as the central nervous systems, and that it also penetrates the blood-brain-barrier to a greater extent than previously thought. Overall, these data provide further compelling pharmacological rationale for the recently reported positive phase 3 interim analysis²."*

Below is the summary of new preclinical findings presented at the meeting that support the use of masitinib in ALS:

New data show protective effects of masitinib in the peripheral nervous system of SOD1^{G93A} (ALS) rats in a therapeutic setting (after paralysis onset).

Masitinib has been shown to generate protective effects in the sciatic nerve:

- Strong upregulation of CSF1 and IL-34 in the degenerating sciatic nerve has been observed for the first time
- A high infiltration of macrophages and a moderate infiltration of mast cells was also observed in the degenerating sciatic nerve
- Masitinib was seen to delay motor axon degeneration in the sciatic nerve
- Masitinib reduces pathological changes in the sciatic nerve, with a sharp decrease of inflammatory infiltrates of CSF1R-expressing macrophages and c-Kit expressing mast cells.

Masitinib has been shown to generate protective effects at the neuromuscular junction (NMJ). It is at the NMJ that the nerve fiber is able to transmit a signal to the muscle fiber, causing muscle contraction:

- Masitinib was seen to delay NMJ denervation in fast skeletal muscles
- Masitinib reduces pathological changes in the NMJ, with a decrease of inflammatory cells in these muscles

Reference:

1. Trias E, et al. Post-paralysis tyrosine kinase inhibition with masitinib abrogates neuroinflammation and slows disease progression in inherited amyotrophic lateral sclerosis. *Journal of Neuroinflammation*, 2016; 13:177. doi:10.1186/s12974-016-0620-9.
2. AB Science press release of April 04, 2016, reporting positive phase 3 interim analysis (<http://www.ab-science.com/en/news>).

About Amyotrophic Lateral Sclerosis

Amyotrophic lateral sclerosis is a rare degenerative disorder that results in progressive wasting and paralysis of voluntary muscles. There are approximately 50,000 people with ALS in the European Union and in the US, with more than 16,000 new cases diagnosed each year in Europe and in the US. Almost 80% of ALS patients die within 5 years and 90% die within 10 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 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 thirteen phase 3 studies in human medicine in metastatic prostate cancer, metastatic pancreatic cancer, relapsing metastatic colorectal cancer, relapsing metastatic ovarian cancer, first-line GIST, second-line GIST, metastatic melanoma expressing JM mutation of c-Kit, relapsing multiple myeloma, relapsing T-cell lymphoma, severe asthma, amyotrophic lateral sclerosis, Alzheimer's disease and progressive forms of multiple sclerosis. 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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