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AB Science has been invited at four international meetings to present new preclinical data showing neuroprotective effect of masitinib in amyotrophic lateral sclerosis (ALS)

New mechanistic data corroborate medical plausibility for using masitinib in the treatment of ALS

AB Science SA (NYSE Euronext – FR0010557264 – AB), a pharmaceutical company specialized in the research, development and marketing of protein kinase inhibitors (PKIs), announces it has been invited to present at four international meetings during 2016 abstracts reporting on new preclinical studies of masitinib in the treatment of amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease.

➤ **Key findings from this series of preclinical studies**

Overall, these data establish medical plausibility for using masitinib in the treatment of ALS.

- **New preliminary data showing masitinib penetrates the blood-brain-barrier to a greater extent than previously thought.**
- **New preliminary data showing that masitinib reduces inflammation in both central and peripheral nervous systems.**
- **Proof of concept that masitinib targets neurotoxic aberrant glial cells via CSF1R inhibition, providing a neuroprotective effect in the therapeutic (post-paralysis) setting and slowing down neurodegeneration.**

➤ **Abstracts and schedule**

Details for each presentation and meeting are listed below.

- **Post-paralysis treatment with masitinib significantly slows down disease progression in transgenic SOD1^{G93A} (ALS) rats**
 - Meeting: 27th International Symposium on ALS/MND
 - Location: Dublin, Ireland (December 7 – 9, 2016)
 - Presentation: Friday, December 9th from 10:30 to 12:40
 - Presenter: Professor Luis Barbeito (Head of the Neurodegeneration Laboratory, Institut Pasteur in Montevideo, Uruguay). Platform Presentation.

The ALS/MND symposium 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. Each year it brings together leading international researchers and health and social care professionals to present and debate key innovations in their respective fields.

- **Masitinib significantly slows down disease progression in postparalysis transgenic SOD1^{G93A} (ALS) rats and reduces inflammation in both central and peripheral nervous systems**
 - Meeting: 15th Annual NEALS Meeting
 - Location: Clearwater Beach, Florida, US (October 5 – 7, 2016)
 - Presentation: Thursday, October 6th from 4:00 to 6:15 PM
 - Presenter: Professor Luis Barbeito (Head of the Neurodegeneration Laboratory, Institut Pasteur in Montevideo, Uruguay)

The Annual NEALS Meeting serves as an opportunity for leading ALS and motor neuron disease scientists, government sponsors, academic partners, and pharmaceutical companies to discuss potential treatments and to share scientific updates.

▪ **Post-paralysis tyrosine kinase inhibition with masitinib abrogates neuroinflammation and slows down disease progression in inherited amyotrophic lateral sclerosis**

Meeting: 2nd Congress of the Federation of Latin-American and Caribbean Societies for Neuroscience (FALAN)

Location: Buenos Aires, Argentina (October 17 – 20, 2016)

Presentation: Thursday, October 20th (Poster session 3)

Presenter: Dr Emiliano Trias (Lead Investigator, Neurodegeneration Laboratory, Institut Pasteur in Montevideo, Uruguay)

▪ **A rationale for post-paralysis tyrosine kinase inhibition [by masitinib] in ALS therapy**

Meeting: 12th Andre-Delambre Annual Symposium on ALS

Location: Quebec City, Canada (September 16 – 17, 2016)

Presenter: Professor Luis Barbeito (Head of the Neurodegeneration Laboratory, Institut Pasteur in Montevideo, Uruguay). Invited Speaker.

➤ **Comment**

“Beyond what has been recently published in the scientific literature¹, we continue to develop our understanding of how masitinib generates the observed neuroprotective effect in ALS” commented Professor Olivier Hermine, President of the Scientific Committee of AB Science. *“For example, in addition to masitinib’s observed neuroprotective effect on the central nervous system we now have ALS model data demonstrating it can regulate neuroinflammation in the peripheral nervous system. Moreover, new preliminary data show that masitinib penetrates the blood-brain-barrier to a greater extent than previously thought, a finding that is also of importance for its development in other neurodegenerative indications, such as Alzheimer’s disease². Overall, these data provide a robust pharmacological rationale for the treatment of ALS with masitinib, while equally conveying plausibility to the recently reported positive phase 3 interim analysis³.”*

“Tyrosine kinase inhibition with masitinib appears unique among other ALS-developmental drugs because it exerts neuroprotection when administrated post-paralysis” said Professor Luis Barbeito (Head of the Neurodegeneration Laboratory, Institut Pasteur in Montevideo, Uruguay). *“One advantage of using models in an advanced therapeutic setting is to closely simulate the clinical condition of ALS patients and their therapeutic needs, thereby increasing the likelihood of replicating the effect in humans. These data therefore provide compelling evidence for masitinib’s therapeutic potential in ALS.”*

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. Folch J, et al. Masitinib for the treatment of mild to moderate Alzheimer's disease. *Expert Rev Neurother*. 2015 Jun;15(6):587-96. doi: 10.1586/14737175.2015.1045419.
3. AB Science press release of April 04, 2016 (<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 twelve 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, severe asthma uncontrolled by oral corticosteroid, 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's website: www.ab-science.com

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