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Presentation of clinical and preclinical data for masitinib in amyotrophic lateral sclerosis (ALS) at 6 major international meetings

New mechanistic data and external publications support the validity of the masitinib mechanism of action in ALS and other neurodegenerative diseases

AB Science SA (NYSE Euronext – FR0010557264 – AB), a pharmaceutical company specialized in the research, development and marketing of protein kinase inhibitors (PKIs), announces today that abstracts reporting on clinical and preclinical data of masitinib in the treatment of amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease, have been selected for oral presentation at 6 major international meetings during 2017, including the World Congress of Neurology held in Japan.

➤ **List of congresses attended**

| Congress | Location | Date |
|---|-----------------------|------------------------|
| XXIII World Congress of Neurology (WCN 2017) | Kyoto, Japan | 19 September, 2017 |
| 16 th Annual NEALS Meeting | Clearwater Beach, USA | 04 October, 2017 |
| 142 nd American Neurological Association (ANA) Annual Meeting | San Diego, USA | 15 & 16 October, 2017 |
| 69 th Spanish Society of Neurology (SEN) Annual Meeting | Valencia, Spain | 04 November, 2017 |
| World Federation of Neurology Research Group on ALS/MND Satellite Symposium | Boston, USA | 07 December, 2017 |
| 28 th International Symposium on ALS/MND | Boston, USA | 08 & 09 December, 2017 |

➤ **New mechanistic data**

○ **New preclinical data support the mechanism of action of masitinib in ALS**

New preclinical data show how the observed treatment effect of masitinib in ALS patients is likely to involve synergistic protective effects in both central and peripheral nervous systems; masitinib targeting both deleterious glial and immune cells expressing tyrosine kinase receptors.

- New data link mast cells to neuromuscular junction denervation during progression of paralysis. This represents a novel pathogenic mechanism in ALS that can be therapeutically targeted by masitinib.
- Masitinib prevented morphological changes in Schwann cells and capillary networks that are typically observed in advanced paralysis.
- New data show that masitinib prevents pathological changes in the degenerating sciatic nerve.

○ **A new study published in Nature validates that microglia, which is the target of masitinib in ALS, is responsible neuronal death in neurodegenerative diseases**

As previously reported in the *Journal of Neuroinflammation*, masitinib generates a neuroprotective effect in a relevant animal model of ALS through targeting aberrant microglial cells and regulating neuroinflammation¹. These data have been recently corroborated by independent research published in *Nature*, identifying that somatic mutations in microglia in mice model is a cause of neuronal death in neurodegenerative diseases², meaning that aberrant microglial cells are drivers of neurodegenerative disease including sporadic ALS.

- **A new independent review underscores the potential of masitinib in neurological and neurodegenerative diseases**

Mast cells in neuroinflammation and brain disorders, published in the journal *Neuroscience and Biobehavioral Reviews* (August 2017)³, reviews a growing appreciation for the importance of mast cell interactions in the brain and their role in the context of neurodegeneration and blood-brain barrier permeability. The potential of masitinib in neurological disorders such as Alzheimer's disease, ischemic brain stroke, and depression in patients with mastocytosis is prominently featured. This broadly corroborates the development program of masitinib in neurology, which in part targets interactions between mast cells and the resident cells of the central nervous system.

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. Mass E, et al. A somatic mutation in erythro-myeloid progenitors causes neurodegenerative disease. *Nature*. 2017 Aug 30. doi: 10.1038/nature23672.
3. Hendriksen E, et al. Mast cells in neuroinflammation and brain disorders. *Neurosci Biobehav Rev*. 2017 Aug;79:119-133.

➤ **Reminder of the key findings from the clinical phase 3 study**

Masitinib orally administered at 4.5 mg/kg/day as an add-on to riluzole demonstrated benefit in ALS patients with a baseline ALSFRS-R progression rate of <1.1 points/month, which was the subgroup pre-specified for the primary analysis and accounting for 85% of the ALS population.

- Significant (p<0.05) 27% slowing of ALSFRS-R deterioration (primary endpoint)
- Significant 29% slowing of deterioration in quality-of-life (ALSAQ-40)
- Significant 22% slowing of deterioration in respiratory function (FVC)
- Significant 25% delay in disease progression (survival-to-event analysis)
- Safety was acceptable

Details for each presentation and meeting

▪ **XXIII World Congress of Neurology (WCN 2017)**

Location: Kyoto, Japan
 Presentation date: 19 September, 2017
 Presenter: Dr. Jesús S. Mora (Director ALS Unit at Hospital San Rafael Madrid, Spain)
 Platform: Oral
 Topic: Masitinib as an add-on therapy to riluzole is safe and effective in the treatment of ALS

The World Congress of Neurology is the world's largest and most important international neurology event. Thousands of participants from around the globe are expected to attend this major scientific meeting, which brings together the world's scientific experts to catalyze and advance scientific knowledge about Neurology.

▪ **16th Annual NEALS Meeting**

Location: Clearwater Beach, Florida, US
 Presentation date: 04 October, 2017
 Presenter: Dr Angela Genge (Medical Director of the Clinical Research Unit at the Montreal Neurological Institute)
 Platform: Oral
 Topic: Masitinib in the treatment of amyotrophic lateral sclerosis

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.

▪ **142nd American Neurological Association (ANA) Annual Meeting**

Location: San Diego, USA
Presentation dates: 15 & 16 October, 2017
Presenter: Dr Angela Genge (Medical Director of the Clinical Research Unit at the Montreal Neurological Institute)
Platform: Poster & Oral (Special Interest Group Session)
Topic: Masitinib in the treatment of amyotrophic lateral sclerosis

The American Neurological Association Annual Meeting is designed to contribute to the overall education of neurologists and neuroscientists. It aims to highlight groundbreaking conceptual and therapeutic advances in a variety of neurologic disease states.

▪ **69th Spanish Society of Neurology (SEN) Annual Meeting**

Location: Valencia, Spain
Presentation date: 04 November, 2017
Presenter: Dr. Jesús S. Mora (Director ALS Unit at Hospital San Rafael Madrid, Spain)
Platform: Oral
Topic: Resultados positivos del ensayo multinacional fase III iniciado en España

It is estimated that this year's annual meeting of the Sociedad Española de Neurología will be attended by more than 3,000 national and international experts, with more than 1200 communications presented on the latest advances in the neurological field.

▪ **World Federation of Neurology Research Group on ALS/MND Satellite Symposium (at the 28th International Symposium on ALS/MND)**

Location: Boston, USA
Presentation date: 07 December, 2017
Presenter: Dr Angela Genge (Medical Director of the Clinical Research Unit at the Montreal Neurological Institute)
Platform: Oral (invited)
Topic: Masitinib: an emerging treatment in ALS

The World Federation of Neurology (WFN) is an association of national neurological societies representing 119 neurological societies in all regions of the world.

▪ **28th International Symposium on ALS/MND**

Location: Boston, USA
Presentation dates: 08 & 09 December, 2017
Presenter: Professor Luis Barbeito (Head of the Neurodegeneration Laboratory, Institut Pasteur in Montevideo, Uruguay)
Platform: Poster
Topic: Masitinib prevents sciatic nerve and sensory afferent pathology in a SOD1^{G93A} rat model of amyotrophic lateral sclerosis

Presenter: Dr Emiliano Trias (Institut Pasteur in Montevideo, Uruguay).
Platform: Poster
Topic: A novel mast cell related pathogenic mechanism in the SOD1^{G93A} rat model of ALS that can be therapeutically targeted by masitinib

Presenter: Dr. Jesús S. Mora (Director ALS Unit at Hospital San Rafael Madrid, Spain)
Platform: Oral
Topic: Masitinib as an add-on therapy to riluzole is safe and effective in the treatment of ALS

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.

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 and is developed in twelve phase 3 indications in human medicine in metastatic prostate cancer, metastatic pancreatic cancer, relapsing metastatic colorectal cancer, relapsing metastatic ovarian cancer, GIST, metastatic melanoma expressing JM mutation of c-Kit, relapsing T-cell lymphoma, mastocytosis, 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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