



## PRESS RELEASE

### **AB SCIENCE SUMMARIZES THE PRESENTATION ON MASITTINIB IN AMYOTROPHIC LATERAL SCLEROSIS MADE AT THE AMERICAN ACADEMY OF NEUROLOGY 2023 ANNUAL MEETING, INCLUDING LONG-TERM SURVIVAL ANALYSIS AND NEW ANALYSIS OF PATIENT POPULATION WITH NO COMPLETE LOSS OF FUNCTION AT BASELINE**

*Paris, 27 April, 2023, 8.30pm CET*

**AB Science SA** (Euronext – FR0010557264 – AB) today summarizes the presentation that was made on masitinib in amyotrophic lateral sclerosis (ALS) at the *American Academy of Neurology (AAN) 2023 Annual Meeting* in Boston, USA (April 22-27, 2023). The AAN Annual Meeting is the world's premier neurology meeting, attracting over 10,000 neurology professionals from around the globe.

The title of this presentation is 'Masitinib Shows Prolonged Survival in Amyotrophic Lateral Sclerosis (ALS) Patients with Mild or Moderate Disease Severity at Baseline'. The abstract will be published in an online supplement to the journal *Neurology*. This presentation is also available on AB Science website ([available here](#)).

The new analysis that was presented was performed in ALS patients prior to any complete loss of physical function (corresponding to a score of 0 on any ALSFRS-R individual component and regardless of baseline progression rate), which encompassed about 80% of the overall AB10015 study population. It is clinically relevant to analyze this population as patients with very severe ALS (i.e. with ALSFRS-R score of zero on any ALSFRS-R individual component) are unable to perform certain physical functions and in the context of treating neurodegenerative disease, any improvement in these lost functions is beyond what can be reasonably expected from an interventional drug, no matter how effective it may be in preventing further disease progression. The exclusion of these very severe patients is also consistent with the proposed mechanism of action of masitinib on microglia and mast cell activity to slow progression rather than as a cure for ALS. Based on this mechanism of action, it would be inappropriate to target those in the last stages of their disease.

Results in these patients prior to any complete loss of physical function showed a statistically significant 18.4% relative benefit on CAFS endpoint in favor of masitinib 4.5 mg/kg/day as compared with control ( $p=0.035$ ). The composite endpoint of functioning and survival (CAFS) was not a recommended primary endpoint when study AB10015 was designed. However, since completion of study AB10015, the CAFS has become increasingly requested as a primary endpoint to determine efficacy in ALS trials, by FDA and Health Canada in particular. This new result therefore represent a key analysis for study AB10015.

The positive treatment-effect in CAFS was supported by converging results on change in ALSFRS-R score at week 48 (+25% difference,  $p=0,027$ ), respiratory function at week 48 (+20.4% difference,  $p=0,022$ ) and quality of life at week 48 (19.8% difference,  $p=0,025$ ). In addition, with long-term survival follow-up (median follow-up of 75 months), there was a close significant median overall survival benefit of +8 months in favour of masitinib 4.5 mg/kg/day (46 [ 30; 69 ] vs 38 [ 29; 49 ],  $p$ -value=0.0684).

Furthermore, patients with moderate disease only (i.e. those patients having ALSFRS-R score greater than 1 on any ALSFRS-R individual component and no fast disease progression) showed even better survival, prolonged of 25 months with masitinib, with a reduced risk of death of 44% ( $P<0.05$ ).

As a reminder, the development program of masitinib in ALS comprises a 48-week clinical trial (AB10015), including long-term survival follow-up analysis, and an on-going confirmatory phase 3 trial (AB19001).

#### **About amyotrophic lateral sclerosis**

Amyotrophic lateral sclerosis (ALS) is a fatal motor neuron disorder that is characterized by progressive loss of the upper and lower motor neurons at the spinal or bulbar level. The disease belongs to a group of disorders known as motor neuron diseases, which are characterized by the gradual degeneration and death of motor neurons. In ALS, both the upper motor neurons and the lower motor neurons degenerate or die, and stop sending messages to muscles. The prevalence of ALS in western countries is fairly uniform at 6 per 100,000 persons, corresponding to around 30,000 cases in Europe and 20,000 in the USA. The first drug treatment for ALS, riluzole (Rilutek), was approved in 1995. In Europe, there has been no new treatment approved since riluzole.

#### **About masitinib**

Masitinib is an 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, inflammatory diseases and viral 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](http://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 published by AB Science. 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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