



PRESS RELEASE

RESULTS OF MASITINIB IN ALS SELECTED FOR A SCIENTIFIC PLATFORM PRESENTATION 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

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AB Science SA (Euronext – FR0010557264 – AB) today announced that Professor Albert Ludolph, MD, PhD (Chairman of the Department of Neurology at the University Hospital and Medical Faculty of Ulm), will deliver a presentation on masitinib in amyotrophic lateral sclerosis (ALS) to an audience of key opinion leaders in the field of ALS healthcare, at the up-coming *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*.

Included in this presentation are the long-term survival data that showed a significantly prolonged survival of 25 months in favor of masitinib for patients with non-severe disease severity at baseline. Furthermore, new analyses performed for the regulatory authorities have shown that starting masitinib treatment in ALS patients prior to any complete loss of physical function (corresponding to a score of 0 on each ALSFRS-R individual component) resulted in a significant treatment-effect across multiple endpoints at week 48, including the joint rank analysis of function and survival (CAFS), which is a key regulatory endpoint.

Of note, this latter population includes all patients regardless of baseline progression rate and encompasses about 80% of the overall AB10015 study population. Moreover, exclusion of patients with a complete loss of function is justified in the context of treating neurodegenerative disease, any improvement in lost function being beyond what can be reasonably expected from an interventional drug, no matter how effective it may be in preventing further progression.

Professor Olivier Hermine, MD, PhD (President of the Scientific Committee of AB Science and member of the Académie des Sciences in France) said, *"Once a clinical study has met its primary objective, it is methodologically acceptable to identify populations that enrich benefit-risk. Furthermore, retrospective analyses based on overall survival are also acceptable, as this is a highly robust endpoint. Beyond the already known increased long-term survival data from patients with moderate ALS severity [2], masitinib has now been shown to produce a significant treatment-effect on the key regulatory accepted outcome of CAFS, provided that treatment starts prior to any complete loss of physical functionality."*

Professor Albert Ludolph commented that, *"These results, including long-term survival data, suggest that masitinib can produce a significant treatment-effect provided that treatment starts early in the disease course of ALS and more importantly prior to any complete loss of physical function. This observation is consistent with mechanistic evidence that masitinib conserves neuro-muscular function as opposed to repairing existing neurological damage. Hence, the current confirmatory phase 3 study (AB19001) is well-aligned with the ALS patient population that is expected to derive greatest benefit from masitinib."*

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). Study AB10015 previously showed that masitinib (4.5 mg/kg/day) as an add-on to standard riluzole, significantly slowed functional decline at week 48 in patients with an ALSFRS-R progression rate of less than 1.1 points/month at baseline, relative to those treated with riluzole alone [1]. Furthermore, long-term follow-up analysis of this population showed a significantly prolonged survival of 25 months for masitinib treated patients that had moderate ALS at baseline [2]. The development of masitinib in ALS is also supported by a well-demonstrated mechanism of action using a relevant model [3–7].

References

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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.

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