



Paris, 18 May 2017, 4.30pm

AB Science presents phase 3 data for masitinib in amyotrophic lateral sclerosis (ALS) at the European Network for the Cure of ALS (ENCALS) annual meeting

Study was a success showing masitinib to be a safe and effective treatment in ALS

AB Science SA (NYSE Euronext - FR0010557264 - AB), a pharmaceutical company specializing in the research, development and commercialization of protein kinase inhibitors (PKIs), today presented the results from its phase 3 trial (AB10015) in masitinib in amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease, at the 2017 ENCALS (European Network for the Cure of ALS) annual meeting (May 18 - 20, 2017, Ljubljana, Slovenia).

Dr. Jesús S. Mora, international coordinator of study AB10015 and neurologist expert in ALS, delivered a plenary presentation (the Thierry Latran Foundation invited lecture) at the ENCALS meeting on Thursday, 18th May. The title of this talk was ***"Masitinib as an add-on therapy to riluzole is beneficial in the treatment of amyotrophic lateral sclerosis (ALS) with acceptable tolerability: Results from a randomized controlled phase 3 trial."***

"These final data confirm findings from the study's interim analysis, showing that masitinib is effective in slowing down ALS disease progression", said Dr. Jesús S. Mora (International coordinator of study, Director ALS Unit at Hospital San Rafael Madrid, Spain). "This benefit is evident from the observed effect on a clinical assessment of daily functioning (Δ ALSFRS-R) and a significant improvement in terms of clinically relevant measures of quality-of-life and respiratory function."

A second, related presentation will be delivered by Professor Luis Barbeito (Head of the Neurodegeneration Laboratory, Institut Pasteur in Montevideo, Uruguay) on Friday, 19th May (08:00-09:00). The title of this talk is ***"Masitinib for the treatment of amyotrophic lateral sclerosis (ALS): Preclinical overview and future clinical development."***

"These phase 3 clinical findings are strongly supported by equally compelling preclinical data, showing masitinib to generate a neuroprotective effect through targeting aberrant microglial cells and regulating neuroinflammation" commented Professor Luis Barbeito. "Indeed, tyrosine kinase inhibition with masitinib appears unique among other ALS-developmental drugs because it exerts neuroprotection in preclinical models from an advanced therapeutic setting."

The full abstracts of both presentations have been published in the ENCALS 2017 conference proceedings and are freely available online from the meeting website: <https://www.encals.eu/meetings/>

➤ **Key features and findings from the masitinib phase 3 study**

Masitinib orally administered at 4.5 mg/kg/day as an add-on to riluzole demonstrated a significant therapeutic benefit with acceptable safety in ALS patients with a baseline ALSFRS-R progression rate of <1.1 points/month.

- Study AB10015 was a phase 3, double-blind, randomized, placebo-controlled trial of masitinib as an add-on to riluzole. ALS patients received riluzole plus masitinib 3.0 mg/kg/day, 4.5 mg/kg/day, or placebo (1:1:1) up to 48 weeks.
- Primary analysis was absolute change of ALSFRS-R_[W0-W48] (Δ ALSFRS-R) in patients receiving masitinib at 4.5 mg/kg/day and with ALSFRS-R progression prior to randomization of less than 1.1 points/month.

- Findings showed that masitinib administered at 4.5 mg/kg/day as an add-on to riluzole in ALS patients experiencing ALSFRS-R progression of <1.1 points/month at baseline, generated a therapeutic benefit when compared with placebo control.
- Significant slowing of disease was evident in terms of:
 - Δ ALSFRS-R (between-group difference of 3.4 [95%CI 0.6–6.1], $P=0.0158$) and ALSFRS-R slope (between-group difference of 27%), indicating a slowed loss of function;
 - Time elapsed between treatment initiation and disease progression (PFS) measured by a decline in functional score (Δ ALSFRS-R) of nine points or death ($P=0.0159$), indicating delayed disease progression;
 - ALSAQ-40 score ($P=0.0078$), indicating reduced decline in quality-of-life; and
 - Forced Vital Capacity ($P=0.0332$), which is considered a surrogate measure of overall survival.
- Supportive results for masitinib 3.0 mg/kg/day revealed a dose-related treatment effect with a beneficial trend on Δ ALSFRS-R ($P=0.066$, between-group difference of 2.7 or 24%) and ALSAQ-40 score ($P=0.006$).
- Post-hoc subgroup analysis selecting patients with less than 24-month duration of illness and with score for each ALSFRS item ≥ 2 , as in edavarone trial, showed a greatly enhanced treatment-effect with respect to placebo, with Δ ALSFRS-R of 4.5 or 42% (between-group difference); $P=0.0176$.
- Safety was acceptable with a positive benefit-risk balance.

“These data prove that masitinib is capable of slowing progression in ALS and effectively opens a new paradigm in the treatment of neurodegenerative diseases” said Professor Olivier Hermine, President of AB Science scientific committee. “Masitinib also appears advantageous considering its convenience of use and broad patient applicability; the former being due to its oral route of administration and the latter because relatively few restrictions were placed on patient inclusion for the phase 3 study in terms of symptom severity and duration of illness. Interestingly, when we apply a more restricted ALS population to the masitinib dataset, for example, in those patients with less than 24 months duration of illness, an even greater treatment-effect for masitinib is observed.”

➤ **Webcall**

A webcall on masitinib in ALS will be hosted in English on May 24, 2017 from 5.30pm to 6.30pm (Paris time). To participate please send an email at linda.carlet@ab-science.com

➤ **Orphan drug status**

Masitinib has been granted orphan drug status in ALS by both FDA and EMA.

➤ **Targeted population with masitinib in ALS**

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 50% of ALS patients die within 3 years and 90% die within 5 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, GIST, metastatic melanoma expressing JM mutation of c-Kit, 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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