Paris, March 31, 2020, 5.30pm

FDA clears masitinib IND in Amyotrophic Lateral Sclerosis (ALS), allowing U.S. patient enrollment to commence in Phase 3 study

AB Science SA (NYSE Euronext - FR0010557264 - AB) today announces that the U.S. Food and Drug Administration (FDA) has cleared the company's Investigational New Drug (IND) application, allowing the Company to initiate its masitinib Phase 3 study (AB19001) in amyotrophic lateral sclerosis (ALS).

"That is really a good news for all of us and in particular for the patients and we aim to initiate the Phase 3 confirmatory trial in ALS as soon as conditions at U.S. clinical sites stabilize post-the coronavirus pandemic," said Alain Moussy, co-founder and CEO of AB Science.

Study AB19001 is an international, multicenter, randomized, double-blind, placebo-controlled, 3-parallel group, Phase 3 study to compare the efficacy and safety of masitinib in combination with riluzole versus placebo in combination with riluzole for the treatment of patients suffering from ALS.

The study's primary endpoint is the absolute change from baseline in functional score as assessed using the Amyotrophic Lateral Sclerosis Functional Rating Scale-revised (ALSFRS-R) after 48 weeks of treatment. The main secondary endpoint is the Combined Assessment of Function and Survival (CAFS).

The study will enroll 495 patients randomized to one of three treatment groups in a 1:1:1 ratio.

- Group 1: Masitinib titration starting at 3.0 mg/kg/day and escalating to 4.5 mg/kg/day, plus riluzole
- Group 2: Masitinib titration starting at 3.0 mg/kg/day and escalating to 4.5 mg/kg/day and then to 6.0 mg/kg/day, plus riluzole
- Group 3: Matching placebo, plus riluzole

Study AB19001 is intended to confirm the previously published results [1] from the first Phase 2/3 study (AB10015) which demonstrated that masitinib at 4.5 mg/kg/day in combination with riluzole significantly slowed ALSFRS-R decline by 27% compared to riluzole alone at week 48 (p-value < 0.05).

Evidence of a dose-response was observed in the previous AB10015 study with maintenance doses of 3.0 and 4.5 mg/kg/day, with an acceptable safety profile. Therefore, this confirmatory trial will evaluate an even higher 6.0 mg/kg/day dose in one of the two active arms.

The design of AB19001 Phase 3 trial has benefited from protocol assistance, the special form of scientific advice available for developers of designated orphan medicines, obtained from the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency's (EMA).

The rationale to use masitinib in ALS patients is supported by a strong body of evidence demonstrating that the pharmacological action of masitinib in microglia and mast cells can slow microglial-mediated disease progression, reduce neuro-inflammation, and modulate the degenerative neuronal microenvironment in both central (CNS) and peripheral nervous systems (PNS) [2–5].

Prof Albert Ludolph, Professor of Neurology and Chairman of the Department of Neurology at the University Hospital and Medical Faculty of Ulm in Germany, and international coordinator of study AB19001 said *"I am extremely pleased with this FDA clearance, which represents a significant milestone for the development of masitinib in ALS. Masitinib is the first drug that targets both microglia and mast cells to be evaluated in ALS.*

I am convinced that this approach represents a promising potential addition to our currently limited therapeutic options for patients with ALS."

References

[1] Mora J, et al. Masitinib as an Add-on Therapy to Riluzole in Patients with Amyotrophic Lateral Sclerosis: A Randomised Clinical Trial. Amyotroph Lateral Scler Frontotemporal Degener. 2019 Jul 7:1-10. https://doi.org/10.1080/21678421.2019.1632346.

[2] Trias E, et al. Schwann cells orchestrate peripheral nerve inflammation through the expression of CSF1, IL-34, and SCF in amyotrophic lateral sclerosis. Glia. Glia. 2019 Dec 20. https://doi.org/10.1002/glia.23768.

[3] Trias E, et al. Mast cells and neutrophils mediate peripheral motor pathway degeneration in ALS. JCI Insight. JCI Insight. 2018;3(19):e123249. https://doi.org/10.1172/jci.insight.123249.

[4] Trias E, et al. Evidence for mast cells contributing to neuromuscular pathology in an inherited model of ALS. JCI Insight. 2017;2(20):e95934. https://doi.org/10.1172/jci.insight.95934.

[5] Trias E, et al. Post-paralysis tyrosine kinase inhibition with masitinib abrogates neuroinflammation and slows disease progression in inherited amyotrophic lateral sclerosis. J Neuroinflammation. 2016;13(1):177.

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. Unable to function, the muscles gradually weaken, waste away (atrophy), and have very fine twitches (called fasciculations). Eventually, the ability of the brain to start and control voluntary movement is lost.

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 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.

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, and inflammatory 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.

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.

For additional information, please contact:

AB Science
Financial Communication & Media Relations
investors@ab-science.com