



**AB SCIENCE ANNOUNCES MASITINIB PATENT GRANTED IN JAPAN FOR THE TREATMENT OF AMYOTROPHIC LATERAL SCLEROSIS, STRENGTHENING THE COMPANY'S INTELLECTUAL PROPERTY POSITION UNTIL 2037**

**TO DATE, THIS PATENT HAS BEEN GRANTED IN 12 REGIONS INCLUDING EUROPE, USA, CHINA, ISRAEL AND MOST RECENTLY JAPAN**

*Paris, 18 April, 2023, 6.45pm CET*

**AB Science SA** (Euronext - FR0010557264 - AB) today announced that the patent office of Japan has issued a Notice of Allowance (NOA) for a patent relating to methods of treating amyotrophic lateral sclerosis (ALS) with its lead compound masitinib (JP 2022037132A). As a result, intellectual property protection for masitinib is secured in ALS until 2037.

This NOA follows patent protection previously granted in other major international markets, including Europe (patent EP 3240538), USA (US 10092564), China (ZL201780019760.9), South Korea (KR 10-2293847), Israel (IL 261856), Australia (AU M53001274), Eurasia (EA 201800499), Mexico (MX 390495), Singapore (SG 11201808106Y), New Zealand (NZ 745778), and South Africa (ZA 2018/05810).

An NOA is issued after an examiner determines that a patent application satisfies all requirements for patentability. This newly issued patent further enhances the Company's key patent family for masitinib, which includes U.S. patents US7423055B2, US8835435B2, US8993573B2, US8153792B2 and US20100093750A1 (as well as their foreign counterparts).

More specifically, this patent provides broad protection of masitinib and related compounds of its class for treatment of ALS in a patient subpopulation initially selected for treatment based on disease aggressiveness (as measured by functional progression rate). This patient population is fully consistent with the clinical development program of masitinib in ALS (study AB10015) including the ongoing international phase 3 randomized clinical trial (AB19001).

Masitinib has also received orphan drug designation for ALS from both the European Medicines Agency (EMA) and the US Food and Drug Administration (FDA). This orphan drug designation provides 10 and 7 years of market exclusivity in Europe and the United States respectively, subsequent to product approval.

Alain Moussy, CEO and co-founder of AB Science said, *"We continue to strengthen the intellectual property portfolio of our lead compound masitinib and confirm the company's strategic commitment in developing innovative drugs that target diseases with high unmet medical needs. This new patent provides strong protection for masitinib in the treatment of ALS until 2037 in the key geographic areas where masitinib could be marketed."*

**About study AB10015**

Study AB10015 was a randomized, placebo-controlled phase 2/3 clinical trial. The aim of this study was to assess the efficacy and safety of masitinib at two different doses (4.5 or 3.0 mg/kg/day) when given as an add-on therapy to riluzole during 48 weeks, as compared with placebo given as an add-on therapy to riluzole, in patients with ALS. This study used a prospectively stratified design based on Amyotrophic Lateral Sclerosis Functional Rating Scale – Revised (ALSFRS-R) progression rate calculated from disease-onset to baseline ( $\Delta$ FS). A cut-off at 1.1 points/month distinguished between 'Normal Progressor' ( $\Delta$ FS<1.1) and 'Fast Progressor' ( $\Delta$ FS $\geq$ 1.1) patients. The primary efficacy endpoint was the change in ALSFRS-R from baseline to week 48 between treatment groups in the 'Normal Progressor' population.

The prespecified primary efficacy analysis on patients receiving masitinib 4.5 mg/kg/day with a  $\Delta$ FS of less than 1.1 points/month ('Normal Progressors') showed a significant benefit over placebo with a between-group difference in

$\Delta$ ALSFRS-R of 3.4 points (-9.2 vs. -12.6);  $p=0.016$ . This corresponds to a 27% slowing in the rate of functional decline. Sensitivity analyses based on multiple imputation, imputing all data at week 48 for all patients with early discontinuation, and multiple imputation with jump-to-reference, treating early discontinuation in the masitinib group as if receiving placebo from the time of discontinuation, remained significant ( $\Delta$ ALSFRS-R of 3.4 points;  $p=0.020$  and  $\Delta$ ALSFRS-R of 2.8 points;  $p=0.039$ , respectively).

A sensitivity analysis was performed in patients with moderate ALS (baseline ALSFRS-R score  $\geq 2$  on each individual component of the ALSFRS-R and  $\Delta$ FS $<1.1$ ), which corresponds to selection of ALS patients early in the course of their disease and is also consistent with newly diagnosed patients. In this cohort, there was a significant 44% reduced risk of death (between group difference in median OS of +25 months, log rank  $p=0.0478$ ; hazard ratio 0.56 (95%CI [0.32;0.96], Cox  $p=0.036$ ) with long-term survival follow-up. The benefit was already apparent at the end the study's blinded treatment period, with a significant 65% reduced risk of death (hazard ratio 0.35 (95%CI [0.13;0.95], Cox  $p=0.039$ )).

### **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.

### **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.

**For additional information, please contact:**

### **AB Science**

Financial Communication & Media Relations

[investors@ab-science.com](mailto:investors@ab-science.com)