



**AB SCIENCE RECEIVES NOTICE OF ALLOWANCE FOR UNITED STATES PATENT COVERING MASITINIB UNTIL 2040 IN THE TREATMENT OF SICKLE CELL DISEASE**

*Paris, April 14, 2025, 6pm CET*

**AB Science SA** (Euronext - FR0010557264 - AB) today announced that the United States Patent Office has issued a Notice of Allowance for a patent relating to methods of treating sickle cell disease (i.e. a medical use patent) with its lead compound masitinib, based on preclinical data. This new US patent provides intellectual property protection for masitinib in this indication until November 2040 and further strengthens masitinib's intellectual property, following a notice of allowance received from the European Patent Office in October 2024 for the same patent.

Professor Olivier Hermine, President of the AB Science Scientific Committee and member of the French Academy of Sciences and Head of Hematology Department at Necker Hospital commented: *"Masitinib is quite unique in the therapeutic landscape of sickle cell disease because no other development drug targets mast cells. Masitinib therefore represents a promising novel strategy for treating sickle cell disease and its serious complication of acute chest syndrome, which is a common cause of hospitalization or even death"*.

Sickle cell disease (SCD) is a group of inherited red blood cell disorders, with masitinib being developed to treat the severest forms of the disease, accounting for about 65% of SCD. Severe SCD represents a major public health challenge and leads to early death. Treatment for SCD can be curative based on gene therapy (targets the HbS mutation), however, such an option remains extremely limited due to scarcity of donors, unresolved safety challenges, and very high costs. Standard treatment for SCD includes red blood cell transfusions and treatment with hydroxyurea to manage complications, but significant unmet need remains.

Mast cells, a major target for masitinib, appear to play a critical role for the severe forms of SCD and its complications, such as vaso-occlusive crises (VOC), acute chest syndrome (ACS), and pain [1-2]. Masitinib has demonstrated survival benefit in an SCD mouse model: all control SCD mice experienced VOC and 83% died in the first 3 hours, whereas SCD mice pretreated with masitinib for 4 days, experienced no VOCs and no death. Furthermore, lung histology and immune immunohistochemistry showed that masitinib protects from acute lung injuries and mast cell infiltration in an SCD mouse model.

Masitinib clinical development in SCD is being conducted as part of the SICKMAST collaborative program, funded with 9.2 million euros [3], which aims to demonstrate in a phase 2 clinical trial the efficacy of masitinib in the treatment of acute and chronic complications of SCD in patients identified based on biomarkers. The Assistance Publique-Hôpitaux de Paris (AP-HP) will be the sponsor of these phase 2 studies. AB Science will mainly be involved in supplying masitinib and monitoring masitinib pharmacovigilance data. AB Science remains free to carry out, as it sees fit, any potential phase 3 development following the success of phase 2.

**Sickle cell disease**

Sickle cell disease (SCD) is an autosomal recessive disorder affecting millions of people worldwide. Although life expectancy has increased over the last 20 years, acute and chronic complications still result in comorbidities, high social burden and premature death at around 40 years. Approximately 1.1% of couples worldwide are at risk of having a child with a hemoglobin disorder (sickle cell disease or thalassemia), and 2.3 conceptions per 1,000 are affected by sickle cell disease. Estimates suggest that each year, around 300,000 children are born with sickle cell disease, and this number could reach 400,000 by 2050 [4]. Sickle

cell disease affects over 100,000 children and adults in the United States. In France, approximately 26,000 patients are affected (50% children, 50% adults).

### **Rationale for the use of masitinib in this indication**

Inflammation mediated by innate immune cells and promoting vaso-occlusion has recently been shown to play a major role in sickle cell disease. In particular, our clinical observations and experimental work in mice, have revealed the involvement of mast cells and basophils in complications associated with sickle cell disease:

- The degree of mast cell activation in patients with sickle cell disease may contribute to the heterogeneity of inflammation and chronic and acute complications.
- The potential role of basophils in sickle cell disease has not been studied, however, given their role in various diseases and their ability to release substance P and histamine, they could also play important roles in the pathophysiology of sickle cell disease.

Masitinib is an inhibitor of KIT, LYN, and FYN, three major kinases involved in the activation of mast cells and basophils.

### **Medical need**

The classic view of sickle cell disease pathophysiology involves polymerization of mutated hemoglobin (HbS) leading to red blood cell (RBC) sickling with subsequent hemolytic anemia, painful vaso-occlusive crisis (VOC) and acute chest syndrome (ACS).

Current treatment options such as hydroxycarbamide, chronic transfusion or anti-P-selectin antibodies, do not fully prevent life-threatening acute and chronic complications of sickle cell disease. Allogeneic stem cell transplantation and gene therapy are available only for a minority of patients, are associated with toxicity and are very expensive, which limits their use.

There is a significant medical need to prevent the acute and chronic complications of sickle cell disease.

### **References**

- [1] Allali S, Lionnet F, Mattioni S, et al. Br J Haematol. 2019;186(1):125-129.
- [2] Allali S, Maciel TT, Hermine O, de Montalembert M. Haematologica. 2020;105(2):273-283.
- [3] AB Science Press Release, 27<sup>th</sup> November 2023. <https://www.ab-science.com/the-clinical-development-of-masitinib-in-sickle-cell-disease-is-among-the-19-winning-projects-under-the-sixth-call-for-hospital-iniversity-research-in-health-rhu/>
- [4] Piel FB, Steinberg MH, Rees DC. Sickle Cell Disease. N Engl J Med. 2017;376(16):1561-1573.

### **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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