



**AB SCIENCE PATENT FOR MASITINIB IN THE TREATMENT OF SICKLE CELL DISEASE FORMALLY GRANTED IN THE UNITED STATES WITH A PROTECTION UNTIL 2040**

**THIS DECISION STRENGTHENS MASITINIB'S INTELLECTUAL PROPERTY PORTFOLIO WITH LONG-TERM PROTECTION FOR AN ADDITIONAL INDICATION WITH A HIGH UNMET MEDICAL NEED**

**THE BIOMARKER PHASE 2 IN COOPERATION WITH AP-HP IS FULLY FUNDED**

*Paris, December 22, 2025, 6pm CET*

**AB Science SA** (Euronext - FR0010557264 - AB) today announced that the United States Patent Office has formally granted a patent for methods of treating sickle cell disease (i.e., a medical use patent) using its lead compound masitinib, based on preclinical data. This new US patent (US12,472,164) ensures intellectual property protection for masitinib until November 2040.

▪ Masitinib : An innovation in sickle cell disease

Sickle cell disease (SCD) is a group of inherited red blood cell disorders, with masitinib being developed to treat the most severe forms of the disease, which account for approximately 65% of cases. Severe SCD poses a major public health challenge and often leads to early death. While SCD treatment can be curative through gene therapy (targeting the HbS mutation), this option remains extremely limited due to donor scarcity, unresolved safety challenges, and high costs. Standard treatment for SCD includes red blood cell transfusions and treatment with hydroxyurea to manage complications; however, significant unmet needs persist.

Mast cells, a major target of masitinib, appear to play a critical role in severe forms of SCD and its complications, such as vaso-occlusive crises (VOC), acute chest syndrome (ACS), and pain [1-2]. Masitinib has demonstrated a 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 immunohistochemistry showed that masitinib protects against acute lung injury and mast cell infiltration in an SCD mouse model.

▪ Biomarker Phase 2 clinical development fully financed

Masitinib clinical development in SCD is being conducted as part of the SICKMAST collaborative program, funded with 9.2 million euros, 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) is the sponsor of this phase 2 study, designed in two steps:

- Part 1 : Identification and validation biomarkers highlighting the role of mast cells and basophils in orchestrating acute and chronic complications of sickle cell disease
- Part 2 : Demonstrating in a phase 2 clinical trial the efficacy of masitinib in the treatment of acute and chronic complications of sickle cell disease in patients identified based on biomarkers

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 : a high unmet medical need

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 [3]. 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).

The disease extends far beyond episodic pain crises. Chronic hemolysis, vaso-occlusion, and endothelial dysfunction lead to progressive organ damage affecting the brain (silent infarcts, overt stroke), kidneys, lungs (pulmonary hypertension, acute chest syndrome), and spleen. This cumulative damage reduces life expectancy by 20-30 years even in high-income settings, and patients often develop complications that current therapies cannot reverse.

Current treatment options such as hydroxycarbamide and chronic transfusion do not fully prevent life-threatening acute and chronic complications of sickle cell disease. Allogeneic stem cell transplantation and gene therapy (Casgevy ; Lyfgenia) are available only for a minority of patients, are associated with toxicity and are very expensive (unit cost of several million euros), which limits their use.

Anti-P-selectin antibodies (crizanlizumab ; inclacumab), once considered promising treatment options, have failed to confirm their efficacy. Voxelotor (hemoglobin modifier) that was conditionally approved was withdrawn from the market to increase occurrence of fatal stroke in post-marketing trials.

There is a high need for new therapeutic approach that prevent long-term organ damages associated with the disease.

### **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] 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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