



THE CLINICAL DEVELOPMENT OF MASITINIB IN SICKLE CELL DISEASE, A HIGHLY PREVALENT GENETIC CONDITION, IS AMONG THE 19 WINNING PROJECTS UNDER THE SIXTH CALL FOR "HOSPITAL-UNIVERSITY RESEARCH IN HEALTH (RHU)".

THIS PROGRAM WILL FUND A MASITINIB PHASE 2 CLINICAL TRIAL

AB SCIENCE WILL HOLD A WEBCAST ON THIS RESEARCH PROGRAM ON THURSDAY, NOVEMBER 30, 2023, FROM 3PM TO 4PM CET

Paris, November 27, 2023, 8am CET

AB Science SA (Euronext - FR0010557264 - AB) announces today that a new clinical development program for masitinib in sickle cell disease is among the 19 winning projects to be funded under the sixth call for "Hospital-University Research in Health" (Recherche Hospitalo-Universitaire en santé) projects, which is part of the Future Investments Program.

The "Hospital-University Research in Health" (RHU) call for projects of the Future Investments Program, operated by the National Research Agency, aims to support innovative and large-scale research projects in the field of health. Focused on translational research, i.e., converting results in basic research into results that directly benefit humans, RHU projects involve academic, hospital, and business stakeholders.

For this sixth wave, an international jury reviewed 62 applications based on criteria of scientific quality, innovation, and potential for medical and socio-economic impact. It proposed to select and fund 19 projects covering various therapeutic areas and medical needs. These 19 projects will receive an exceptional grant of 160 million euros from the Future Investments Program.

As part of this call for projects, the SICKMAST project, funded with 9.2 million euros, aims to:

- First, identify and validate, from a database of 1500 patients (including 700 already identified), biomarkers highlighting the role of mast cells and basophils in orchestrating acute and chronic complications of sickle cell disease.
- Second, demonstrate 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.

The Assistance Publique-Hôpitaux de Paris (AP-HP) will be the promoter 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.

A new patent has been filed, which, if granted, will extend the international protection of masitinib in sickle cell disease until 2040. As part of the consortium agreement established for its patents, AB Science will pay royalties to APHP in the event of commercialization of masitinib in sickle cell disease.

Prof. Olivier Hermine, Necker-Enfants malades AP-HP, coordinator of the SICKMAST program, said, *"We are delighted to be among the laureates of this program, recognized for its extremely rigorous selection of innovative and medically impactful projects. Sickle cell disease represents a major public health challenge due to the number of affected individuals and the early mortality it causes. This program should allow us to demonstrate the relevance of selectively targeting two key immune cells in the treatment of this disease and identifying patients most likely to respond through new biomarkers."*

Alain Moussy, President and co-founder of AB Science, said, *"We are very pleased with this collaboration with APHP, one of the largest promoters of clinical studies in France, whose research excellence is globally recognized. This project demonstrates the full potential of masitinib in targeting certain key immune cells involved in numerous pathologies. It opens an extremely promising new development avenue for masitinib and offers partnership prospects that we will explore to enable the registration and commercialization of masitinib in this indication under the best conditions."*

A webcast with experts in sickle cell disease will be held on November 30, 2023, to provide a more detailed presentation of this project. Login details for this live webcast will be provided later.

▪ **Scientific rationale**

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.

▪ **Epidemiology**

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 [1]. 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).

▪ **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.

Reference

[1] 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.

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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