



PRESS RELEASE

AB SCIENCE REPORTS A FIRST COMPLETE BONE MARROW RESPONSE IN A RELAPSED REFRACTORY ACUTE MYELOID LEUKEMIA (AML) PATIENT FROM THE VERY LOW DOSE ARM OF ITS AB8939 PHASE I/II CLINICAL TRIAL (AB18001)

AB SCIENCE WILL HOST A LIVE WEBCAST ON ITS NEW MICROTUBULE PLATFORM ON THURSDAY MARCH 16, 2023, FROM 6PM TO 7PM CET

Paris, 13 March, 2023, 6pm CET

AB Science SA (Euronext – FR0010557264 – AB) today announces a case report from the initial stage of its Phase I/II study (AB18001) evaluating AB8939, a microtubule destabilizer, in patients with refractory and relapsed acute myeloid leukemia (AML).

The AML patient in question was in failure to prior treatment with azacitidine and presented with a MECOM gene rearrangement, which is a biomarker for resistance to standard chemotherapies that is associated with a high-risk of disease progression and inferior prognosis.

One month after the first treatment cycle (i.e., three consecutive days of AB8939 treatment) there was a drastic reduction in bone marrow blast cells (i.e., leukemia cells), from a pretreatment level of 55% to 5% (i.e., a morphologic leukemia free state). Remarkably, this response was achieved at a very low dose of AB8939, corresponding to the second step of dose increment (out of 13 potential steps) in phase I. The patient also showed excellent tolerance to AB8939, having experienced no treatment-related toxicities. At the request of the investigator, AB Science has authorized further treatment cycles of AB8939 to this patient. One month after the second treatment cycle of three consecutive days at this dose, a good response has been maintained with bone marrow blasts being at 10% (corresponding to a 5-fold reduction relative to baseline). A third treatment cycle for this patient has been initiated.

Considering the overall study to date, there have been no signs of moderate, severe or serious toxicity and approximately 50% of patients have requested further treatment cycles of AB8939 after the first cycle of treatment and a measurement at day 28.

Dr Pau Montesinos, hematologist at the La Fe University Hospital and coordinator of the Spanish group of acute myeloblastic leukemia (PETHEMA), said *“It is remarkable that we rapidly observed a response in what is typically a difficult-to-treat patient population of refractory AML. We observe a clear blast count reduction for this patient and excellent tolerance so far. It is all the more noteworthy because the initial disappearance of leukemic cells was obtained after only 3 days of AB8939 treatment at a very low dose, with a good response maintained after a second 3-day cycle at this dose.”*

Professor Olivier Hermine, MD, President of the Scientific Committee of AB Science and member of the Académie des Sciences in France said *“This preliminary clinical data provides the most encouraging signs to date that AB8939 may be well-suited for treatment of high-risk relapsed/refractory AML. AML is a serious life-threatening condition and the most common cause of leukemia-related mortality, in large part because patients develop chemoresistance to existing frontline AML drugs.”*

AML represents a heterogeneous group of diseases with different responses to treatment, which can be separated by genetic abnormalities. Overexpression of MECOM occurs in approximately 10% of AML patients

and is associated with a poor prognosis, in part due to its important role in the maintenance of leukemic stem cells (LSCs) [1]. Because of their quiescent (inactive) state, LSCs are not targeted by any antimitotic chemotherapy and can therefore re-establish the disease after therapy.

AB8939 treatment of this AML subpopulation is the subject matter of a provisional patent application filed by AB Science.

Study AB18001 is titled '*A Phase 1/2 Study to Assess the Safety, Pharmacokinetics, and Efficacy of Daily Intravenous AB8939 in patients with Relapsed/Refractory Acute Myeloid Leukemia*'. The study has a multi-stage design. The first part is a dose escalation study that aims to determine the safety and tolerability of intravenous AB8939 in patients with refractory or relapsed AML or patients with refractory myelodysplastic syndrome (MDS), and to determine the recommended dose for the second-stage dose expansion study. This dose expansion study aims to determine the schedule for a Phase 2 trial in patients with relapsed/refractory AML and to also provide an early efficacy (response rate) assessment of AB8939.

AB Science has developed an in-house, proprietary, platform of new generation synthetic microtubule destabilizer agents (MDA). To date, two of these MDAs have entered its drug development pipeline. AB8939 is being developed for hematological malignancies and is at the early clinical trials stage. A second, orally administered MDA is being developed for oncology indications, and is starting regulatory preclinical studies, which are required to initiate phase 1 clinical trials.

AB Science will host a live webcast on Thursday March 16, 2023, from 6pm to 7pm CET, to present in further detail the synthetic microtubule destabilizers platform and the on-going phase 1/2 with AB8939. Login details for this live webcast will be provided later.

About AB8939

AB8939 is a new synthetic microtubule-destabilizing drug. Preclinical data show that AB8939 has broad anticancer activity [2-4], with a notable advantage over standard chemotherapies that target microtubules of being able to overcome P-glycoprotein (Pgp) and myeloperoxidase (MPO) mediated drug resistance. Development of drug resistance often restricts the clinical efficacy of microtubule-targeting chemotherapy drugs (for example, taxanes and vinca alkaloids); thus, AB8939 has strong potential to be developed in numerous oncology indications.

AB8939 was granted orphan drug designation for AML from the U.S. Food and Drug Administration (FDA).

AB8939 was entirely discovered by the laboratories of AB Science, which retains full ownership of intellectual rights, and is an example of AB Science's focus on innovative drug development focused on improving patients' lives.

The first indication AB8939 is being developed for is acute myeloid leukemia (AML). Cytarabine (Ara-C) and azacytidine are standard chemotherapies for AML treatment, however, drug resistance is a major limitation to successful therapy. *In vivo* data from a highly resistant Ara-C patient derived xenograft (PDX) mouse model showed that AB8939, administered alone or in combination with Ara-C, increased survival relative to single agent Ara-C, with an accompanying significant reduction of blasts in blood and decrease in tumor growth. Further evidence of therapeutic potential was demonstrated using an azacytidine resistant PDX model with AB8939, administered alone or in combination with azacytidine, showing a significant reduction of blasts relative to single agent azacytidine. Moreover, while azacytidine was associated with strong treatment related hematotoxicity, AB8939 did not induce hematotoxicity throughout its 4-week treatment period.

References

[1] Paubelle E, Plesa A, Hayette S, et al. Efficacy of All-Trans-Retinoic Acid in High-Risk Acute Myeloid Leukemia with Overexpression of EVI1. *Oncol Ther.* 2019;**7(2):121-130**. Doi:10.1007/s40487-019-0095-9

[2] Goubard A, Humbert M, Mansfield C, Hermine O, Dubreuil P, et al. *In Vivo Assessment of the Next Generation Microtubule-Destabilizing Agent AB8939 in Patient-derived Xenograft Models of Acute Myeloid Leukemia*. **Blood (2019) 134 (Supplement_1): 5142. doi.org/10.1182/blood-2019-127143**

[3] Goubard A, Humbert M, Mansfield C, Hermine O, Dubreuil P, et al. *AB8939, a Microtubule-Destabilizing Agent with Potential to Overcome Multidrug Resistance, is Active Across the Range (M0–M7) of Acute Myeloid Leukemia Subtypes*. **Blood (2019) 134 (Supplement_1): 5154. doi.org/10.1182/blood-2019-127021**

[4] Humbert M, Goubard A, Mansfield C, Hermine O, Dubreuil P, et al. *Anticancer Activity of a Highly Potent Small Molecule Tubulin Polymerization Inhibitor, AB8939*. **Blood (2019) 134 (Supplement_1): 2075. doi.org/10.1182/blood-2019-122540**

About acute myeloid leukemia (AML)

Acute myeloid leukemia (AML) is a serious, life-threatening condition and the most common cause of leukemia-related mortality, with a majority of patients facing a highly unsatisfactory prognosis. As such, AML represents an unmet medical need, with limited therapeutic options for patients who are refractory or too frail to benefit from potentially curative but highly toxic treatment, or for those patients that have relapsed following a first complete response. The prevalence of AML in western countries is around 1 per 5,000 persons, corresponding to around 100,000 cases in Europe and 60,000 in the USA. Among AML patients, it is estimated that approximately 50% of the patients will not have stem cell transplantation and will relapse. Therefore, the estimated targeted population of AB8938 in AML is around 80,000 people in Europe and the US.

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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