



PRESS RELEASE

AB SCIENCE RECEIVES HEALTH CANADA APPROVAL LETTER TO COMMENCE PHASE I/II TRIAL OF AB8939 IN THE TREATMENT OF ACUTE MYELOID LEUKEMIA (AML)

AB8939 IS A NEW GENERATION SYNTHETIC MICROTUBULE DESTABILIZER WITH THE ABILITY TO OVERCOME MULTIDRUG RESISTANCE AND HAS BROAD APPLICABILITY AS A POTENT ANTICANCER DRUG

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AB Science SA (Euronext - FR0010557264 - AB) today announced that its clinical trial with AB8939 in adult patients with relapsed/refractory acute myeloid leukemia (AML) has been approved by Health Canada. The 'No Objection Letter' (NOL) received from Health Canada provides an acknowledgement of AB8939's drug candidacy and the authority to proceed with a Phase I/II study (AB18001) in patients with refractory and relapsed AML and refractory myelodysplastic syndrome (MDS).

AB8939 is a new generation synthetic microtubule destabilizer with the ability to overcome multidrug resistance and the potential for broad applicability as a potent anticancer drug. Microtubules play a crucial role in multiple cellular functions which makes them an important target for cancer therapy. Indeed, chemotherapies that target microtubules, such as taxanes and vinca alkaloids, are among the most successful anticancer therapeutics available. Unfortunately, the development of drug resistance (for example, via Pgp efflux pumps that transport the drugs out of the cancer cells) often restrict their clinical efficacy.

Key characteristics of AB8939 are that it circumvents difficulties associated with Pgp-dependent multidrug resistance and is not deactivated by an enzyme named myeloperoxidase, which is an advantage over existing chemotherapies. Another advantage and distinguishing characteristic of AB8939 is that it is a synthetic drug.

The therapeutic potential of AB8939 has been demonstrated through a series of preclinical experiments [1–3]. *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 [1]. Ara-C is considered the clinically most relevant cytotoxic drug for AML treatment. In another example, cancerous tumors from patients suffering from resistant acute megakaryoblastic leukemia (an AML subtype) were transplanted into mice. Data showed a complete response in mice treated with AB8939, as compared with rapid disease progression in control animals [2]. No apparent toxicity was observed during the time course of the treatment.

Based on these results, AB8939 was granted orphan drug designation for AML from the U.S. Food and Drug Administration (FDA) [4].

The first indication AB8939 is being developed for is acute myeloid leukemia (AML), a rapid proliferating hematological cancer that originates in the bone marrow and quickly moves into the blood. Cytarabine (Ara-C) is the current standard chemotherapy for AML treatment, however, drug resistance is a major limitation to successful therapy. AB8939 therefore has strong potential as a second or third-line treatment in AML patients who are unfit to receive intensive chemotherapy.

The advantageous mechanistic characteristics of AB8939 mean that it is potentially applicable to a large number of other oncology indications currently treated by microtubule-inhibitor drugs (such as taxanes and

vinca alkaloids) and in particular hematological cancers. The envisioned strategy is to position AB8939 in patients with abnormal cytogenetics that make these patients unresponsive to first-line therapy.

Professor Olivier Hermine, President of the Scientific Committee of AB Science and member of the Académie des Sciences in France said, “*We believe that AB8939 could represent a major breakthrough in the development of effective microtubule-targeting agents, an extensively used class of cancer treatment. Our preclinical data show that AB8939 exhibits broad anticancer activity, with a notable advantage of it being able to overcome common mechanisms of drug resistance. AB8939 therefore has strong potential to be developed in numerous oncology indications, with our initial targets being hematological cancers. We are excited about the start of this first AB8939 study in acute myeloid leukemia (AML), which represents a significant milestone in the clinical program of AB Science. AB8939 seems particularly well-suited for the treatment of relapsed or refractory AML, for which there are very limited therapeutic options.*”

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.

About Study AB18001

Study AB18001, titled ‘*A Phase 1/2 Study to Assess the Safety, Pharmacokinetics, and Efficacy of Daily Intravenous of AB8939 in patients with Relapsed/Refractory Acute Myeloid Leukemia*’, 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 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.

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 [5], 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.

References

[1] 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**

[2] 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**

[3] 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**

[4] Press release dated November 7, 2019

[5] National Cancer Institute (<https://seer.cancer.gov/statfacts/html/amyl.html>)

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.

For additional information, please contact:

AB Science

Financial Communication & Media Relations

investors@ab-science.com

Media Relations – USA

RooneyPartners

Kate Barrette

kbarrette@rooneyco.com

+1 646 432 0191

Media Relations – France

NewCap

Arthur Rouillé

arouille@newcap.fr

+33 (0)1 44 71 00 15