



Paris, July 09, 2019, 8am

## Summary of the July 8 2019 web conference

### Validation of the AB8939 clinical development plan through regulatory authority *Scientific Advice* procedure

### Positive case report with dog suffering from acute lymphoblastic leukemia and treated with AB8939

**AB Science SA** (NYSE Euronext - FR0010557264 - AB) is providing a summary of the web conference held on July 8 2019 on the AB8939 clinical development in Acute Myeloid Leukemia (AML).

#### **AB8939 differentiating points**

AB8939 is a new microtubule destabilizer that differs from other drugs of this class because it is a synthetic, as opposed to being derived from nature, and because it is not transported by the Pgp protein; thereby, overcoming Pgp-dependent multidrug resistance (a problem for many anthracycline drugs that are used in standard AML treatment).

AB8939 is initially being developed in AML because cancer cells proliferate rapidly in this disease. AB8939 is 100 times more potent than doxorubicin (adriamycin), which is a reference drug in AML. Furthermore, AB8939 is not deactivated by myeloperoxidase enzyme, which is an advantage over vinca alkaloids (vincristine or vinblastine).

#### **EMA Scientific Advice procedure**

The strategy is to position AB8939 in patients with abnormal cytogenetics that make these patients unresponsive to first line therapy. The EMA validated the design and the methodology of the upcoming AB8939 phase 1/2 study through a *Scientific Advice* procedure. Specifically, the EMA validated that all non-clinical studies required to initiate clinical studies in patients have been completed, validated the methodology to determine the maximum tolerated dose (MTD), the safety rules and the possibility, depending on the complete response rate observed in phase 1, to launch an early phase 2 study which could potentially support accelerated approval based on complete response rate versus historical control.

#### **AB8939 proof of concept in mice**

A positive AB8939 proof of concept was established in mice. Cancerous tumours from patients suffering from aggressive acute megakaryoblastic leukemia (AMKL) and resistant to doxorubicin were transplanted into mice. Data showed a complete response in mice treated with AB8939, as compared to control. No apparent toxicity was observed during the time course of the treatment.

#### **AB8939 proof of concept in dogs**

A positive AB8939 case report was also obtained in a 12 years old female dog suffering from an acute lymphoblastic leukemia (ALL) in failure to several cycles of standard treatment (vincristine, L-asparaginase, prednisone) and with 90% blast in the bone marrow. The dog was treated for five consecutive days with

AB8839. From day 9, there was a continuous improvement in all hematologic parameters (white blood count, neutrophils, lymphocytes, platelets). After 3 weeks of treatment, the dog was alive and with good appetite and shape, no weight loss, no fever, no edema, and moving easily.

Olivier Hermine, MD, PhD, commented *"ALL is a dramatic condition, in particular in failure to several lines of chemotherapy. These results are very interesting as they suggest that AB8939 has a positive therapeutic index with good tolerance in dogs and suggest efficacy on leukemia stem cells. These results in a natural disease provide valuable information for translation into human medicine"*.

### **Targeted population in AML**

The prevalence of AML in western countries is around 1 per 5,000 persons [1], corresponding to around 100,000 cases in Europe and 60,000 in the USA. Among the 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 in the US.

### **References**

[1] 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, and inflammatory 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 filed by AB Science with the Autorité des Marchés Financiers (AMF), including those listed in the Chapter 4 "Risk Factors" of AB Science reference document filed with the AMF on November 22, 2016, under the number R. 16-078. 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](mailto:investors@ab-science.com)