PRESS RELEASE

EUROPEAN MEDICINES AGENCY (EMA) HAS GRANTED ORPHAN DRUG DESIGNATION IN THE EUROPEAN UNION FOR AB8939 IN THE TREATMENT OF ACUTE MYELOID LEUKEMIA

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AB Science SA (Euronext - FR0010557264 - AB) announces today that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) granted orphan drug status to AB8939 for the treatment of acute myeloid leukemia (AML).

AB8939 had already obtained orphan drug designation from the US Food and Drug Administration (FDA) in AML.

Granting of this orphan drug status in the EU is a significant milestone because it means that the COMP considered that AB8939 offers a significant benefit to people suffering from this condition in addition to existing treatments.

Indeed, the criteria to obtain orphan drug designation at EMA differ from those at FDA and are very stringent for the following reasons :

- There must be no satisfactory method of diagnosis, prevention or treatment of the condition or, if such a method exists, the medicinal product must offer a significant benefit to patients.
- Because the application is based on an assumption of significant benefit, a comparison with authorized treatments is required.
- Significant benefit means that a medicine produces a clinically relevant advantage or makes a major contribution to patient care, as compared with existing methods to treat the condition. Thus, orphan designation is given to a product that will improve patients' current treatment, having considered what else is available.
- To follow the spirit of the orphan legislation, which makes it clear that an orphan application may be made at any stage of the development, significant benefit will be based on the available evidence at the stage of designation.

In support of the significant benefit of AB8939 in AML, AB Science has released preclinical data from mouse models demonstrating a significant benefit of AB8939 treatment over current therapies, such as cytarabine, azacitidine (Vidaza®) and venetoclax (Venclexta®). This included:

- Efficacy on resistant cells: AB8939 manages to have an effect on the cancer cells (blasts) of AML patients, even when these cells are resistant to other drugs such as vincristine or cytarabine. For example, 45% of vincristine-resistant cells and 66% of cytarabine-resistant cells still respond to AB8939, including in severe cases with complex genetic mutations (MECOM, TP53).
- Convincing results in xenograft models derived from refractory AML patients: In these mouse models which mimick human AML, AB8939 reduces tumors and prolongs survival, even when cells are resistant to cytarabine.
- Additive effect with reference treatments: When used with other treatments (cytarabine, Vidaza® or venetoclax), AB8939 further improves results. For example, with venetoclax, it eliminates cancer cells in the blood, spleen and bone marrow, without serious side effects.

- Furthermore, unlike venetoclax, AB8939 does not cause blood toxicity (hematotoxicity) and appears to act synergistically with other treatments, reinforcing its efficacy.

AB Science also presented preliminary efficacy and safety data from phase 1 of AB8939 as a monotherapy, with a 3-day treatment cycle (stage 1 of phase 1) and a 14-day treatment cycle (stage 2 of phase 1).

Professor Olivier Hermine, President of AB Science's Scientific Committee, member of the French Academy of Sciences and Head of the Hematology Department at Necker Hospital, commented*: "This designation testifies to the potential of AB8939 for the treatment of AML. Indeed, AB8939 has shown activity as a monotherapy on Ara-C-resistant patient lines, including in unfavorable genetic situations (MECOM, TP53 mutations) that have resisted all treatments administered to date, as well as a synergistic effect with the reference treatments Vidaza® and Venclexta®. The ongoing Phase 1 trial will now evaluate the combination of AB8939 with these reference treatments in refractory patients".*

About AB8939

AB8939 is a new synthetic molecule which jointly targets cancer cells, by destabilizing the microtubules essential for cell division, and cancer stem cells, by inhibiting enzymes (ALDH1A1 and ALDH2) essential for maintaining their physiological state and survival. The molecule '1-{4-[2-(5-ethoxymethyl-2-methylphenylamino)-oxazol-5-yl]phenyl}imidazolidin-2-one' is the chemical name of AB8939. The intellectual property of AB8939 is 100% owned by AB Science.

About the benefits of orphan drug designation

European orphan drug designation is granted by the European Commission to medicinal products intended for the treatment of life-threatening or chronically debilitating conditions that affect no more than 5 in 10,000 people in the European Union (EU), or for which it must be unlikely that the marketing of the medicinal product will generate sufficient revenue to justify the investment required for its development. In addition, there must be no satisfactory method of diagnosis, prevention or treatment of the condition concerned, or, if such a method exists, the drug must offer a significant benefit to sufferers.

An orphan drug designation in the EU confers a range of benefits on sponsoring companies, including scientific advice on all aspects of product development at a reduced fee, direct access to the centralized marketing authorization procedure, and eligibility for certain financial incentives made available by the Community and Member States to support research and development of orphan drugs. If the product is approved for marketing, the designation also confers 10 years of marketing exclusivity from product registration if the orphan drug designation still prevails at the time of marketing authorization.

In February 2018, the EMA published a Q&A document to clear up common misunderstandings about the meaning of orphan drug designation and other aspects related to orphan drugs:
https://www.ema.europa.eu/en/documents/other/rare-diseases-orphan-medicines-getting-facts-straight_en.pdf

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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