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AB Science announces publication in *The Lancet* of phase 3 clinical trial results demonstrating masitinib is effective and well tolerated in the treatment of adult patients with severe indolent systemic mastocytosis

AB Science SA (NYSE Euronext – FR0010557264 – AB), a pharmaceutical company specialized in the research, development and marketing of protein kinase inhibitors (PKIs), announced today that results from the pivotal phase 3 (AB06006) study of masitinib in severe indolent systemic mastocytosis were published in *The Lancet*, one of the most highly regarded and well known medical journals in the world, following peer review. Masitinib is the first drug to demonstrate efficacy in a phase 3 study of adult patients with severe indolent systemic mastocytosis, including the subvariant of smouldering systemic mastocytosis, who are unresponsive to existing, optimal symptomatic treatments¹.

The paper titled: "Masitinib for treatment of severely symptomatic indolent systemic mastocytosis: a randomised, placebo-controlled, phase 3 study" [Lortholary O, et al. *Lancet* 2017; published online Jan 6 DOI:[http://dx.doi.org/10.1016/S0140-6736\(16\)31403-9](http://dx.doi.org/10.1016/S0140-6736(16)31403-9)], along with associated supplementary material, is accessible from The Lancet website ([www.thelancet.com/journals/lancet/article/PIIS0140-6736\(16\)31403-9/fulltext](http://www.thelancet.com/journals/lancet/article/PIIS0140-6736(16)31403-9/fulltext)).

Below is the summary of the article published in *The Lancet*:

- In this pivotal phase 3 study (AB06006), masitinib administered at 6 mg/kg/day was significantly superior to placebo, as measured by the cumulative 75% response rate until week 24 on the handicaps of pruritus or flushes or depression or fatigue (primary endpoint 4R75%). The 4R75% response was 18.7% for the masitinib treatment-arm versus 7.4% for the placebo treatment-arm ($p=0.0076$)¹.
- Subgroup analysis in patients with the KIT Asp816Val (D816V) mutation (90% of the population) also showed a significant response in favour of masitinib, with a 4R75% response of 20.2% for masitinib versus 7.4% for placebo ($p=0.0316$)¹.
- Treatment response was sustained over a period of 2 years¹.
- Safety assessment showed a comparable incidence of adverse events between treatment-arms¹.
- Masitinib is the first drug to show significant therapeutic benefit across a diverse range of symptoms as well as objective markers of mast cell activation in patients with severe indolent systemic mastocytosis¹.

Based on the phase 3 study results, AB Science filed for registration to the European Medicines Agency (EMA) in April 2016 for masitinib in severe indolent systemic mastocytosis.

Dr Michel Arock from the Ecole Normale Supérieure de Cachan (Paris, France), an expert in mastocytosis commissioned by *The Lancet* to comment on the published article concluded "*These data offer high hopes for masitinib, which could become an attractive alternative to the therapeutic arsenal available for patients with severely symptomatic indolent or smouldering systemic mastocytosis*"².

Alain Moussy, Chief Executive Officer of AB Science said *“Publication of these pivotal phase 3 data in a prestigious medical journal such as The Lancet marks another important milestone in the path to bring masitinib to patients with severely symptomatic indolent systemic mastocytosis, an indication for which there is currently no registered or established standard treatment and a high unmet medical need. Considering that The Lancet sets extremely high standards for peer review and acceptance, its publication of these data is an indication that Key Opinion Leaders consider masitinib as a potential new treatment option for adult patients with severely symptomatic indolent systemic or smouldering systemic mastocytosis, who are unresponsive to existing symptomatic treatments.”*

“The AB06006 study data now published in The Lancet show, for the first time, a new therapy that can substantially reduce severe symptoms associated with indolent systemic mastocytosis, regardless of a patient’s c-Kit mutational status” said Professor Olivier Hermine M.D., senior author of the paper, President of the Scientific Committee of AB Science and coordinator of the Reference Center for Mastocytosis (CeReMast, Paris, France). *“Study AB06006 successfully achieved its primary and secondary objectives; moreover, both efficacy and safety data indicate a possibility for effective long-term management of this difficult-to-treat condition. This observation is important, given that indolent systemic mastocytosis is a chronic condition that requires lifelong management. Masitinib may therefore be an important new treatment option for these patients.”*

[1] Lortholary O, et al. Masitinib for treatment of severely symptomatic indolent systemic mastocytosis: a randomised, placebo-controlled, phase 3 study. *Lancet* 2017; published online Jan 6. DOI:[http://dx.doi.org/10.1016/S0140-6736\(16\)31403-9](http://dx.doi.org/10.1016/S0140-6736(16)31403-9).

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[2] Arock M. A new therapeutic advance for symptomatic systemic mastocytosis? *Lancet* 2017; published online Jan 6. DOI:[http://dx.doi.org/10.1016/S0140-6736\(16\)31655-5](http://dx.doi.org/10.1016/S0140-6736(16)31655-5).

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About The Lancet medical journal

The Lancet is the world's leading independent general medical journal. The journal's coverage is international in focus and extends to all aspects of human health. The Lancet publishes original primary research and review articles of the highest standard. The Lancet is stringently edited and peer-reviewed to ensure the scientific merit and clinical relevance of its diverse content. Drawing on an international network of advisers and contributors, The Lancet meets the needs of physicians by adding to their clinical knowledge and alerting them to current issues affecting the practice of medicine worldwide.

Journal Metrics: The Lancet has an Impact Factor of 44.002 and a 5-Year Impact Factor of 46.119 (2016 Release of Journal Citation Reports®, Thomson Reuters).

About masitinib

Masitinib is a new orally administered tyrosine kinase inhibitor that targets mast cells and macrophages, important cells for immunity, through inhibiting a limited number of kinases. Based on its unique mechanism of action, masitinib can be developed in a large number of conditions in oncology, in inflammatory diseases, and in certain diseases of the central nervous system. In oncology due to its immunotherapy effect, masitinib can have an effect on survival, alone or in combination with chemotherapy. Through its activity on mast cells and microglia and consequently the inhibition of the activation of the inflammatory process, masitinib can have an effect on the symptoms associated with some inflammatory and central nervous system diseases and the degeneration of these diseases.

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 in cancers, inflammatory diseases, and central nervous system diseases, both in humans and animal health.

AB Science has developed a proprietary portfolio of molecules and the Company's lead compound, masitinib, has already been registered for veterinary medicine in Europe and in the USA. The company is currently pursuing thirteen

phase 3 studies in human medicine in metastatic prostate cancer, metastatic pancreatic cancer, relapsing metastatic colorectal cancer, relapsing metastatic ovarian cancer, first-line GIST, second-line GIST, metastatic melanoma expressing JM mutation of c-Kit, relapsing multiple myeloma, relapsing T-cell lymphoma, severe asthma, amyotrophic lateral sclerosis, Alzheimer's disease and progressive forms of multiple sclerosis. 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 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.

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