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## Masitinib as an Adjunct Therapy for Alzheimer's Disease

Publication in *Alzheimer's Research and Therapy*

**AB Science SA** (NYSE Euronext - FR0010557264 - AB), a pharmaceutical company specializing in the research, development and commercialization of protein kinase inhibitors (PKIs), announces the publication of results from the first human phase 2 study of masitinib carried-out in the treatment of Alzheimer's disease. Entitled, '*Masitinib as an adjunct therapy for mild-to-moderate Alzheimer's disease: a randomised, placebo-controlled phase 2 trial*', this article is freely accessible online from BioMed Central's peer-reviewed journal **Alzheimer's Research and Therapy** (<http://alzres.com/content/3/2/16>).

- **Phase 2 study establishes proof-of-concept that oral masitinib has potential therapeutic benefits in patients with mild-to-moderate Alzheimer's disease**
- **Overall, results add new scientific data to the important question of the potential role of anti-inflammatory agents in the management of Alzheimer's disease**
- **AB Science is actively preparing to launch a phase 3 study, pivotal in the process of registration of masitinib in this indication**

This randomized placebo-controlled phase 2 trial, conducted by Professor François Piette (Hôpital Charles Foix in Ivry-sur-Seine) and colleagues from 12 study centers across France, investigated the hypothesis that masitinib's targeted inhibitory action on mast cells may reduce the symptoms of Alzheimer's disease. A total of 35 patients were included in this study. Neuroinflammation is thought to be important in Alzheimer's disease pathogenesis. Mast cells are a key component of the inflammatory network and participate in the regulation of the blood-brain barrier's permeability. Masitinib, a selective oral tyrosine kinase inhibitor, effectively inhibits the survival, migration and activity of mast cells.

Professor Marc Verny (Head of the Geriatric Department, Pitié-Salpêtrière hospital in Paris and study co-investigator) commented: « *Masitinib administered as an add-on therapy to standard care during 24 weeks showed promising signs of retarding the rate of cognitive decline of Alzheimer's disease compared to placebo, with an acceptable tolerance profile. Although the size of this study was too small to make any definitive conclusions about treatment efficacy, the evidence is sufficiently compelling to warrant further phase 3 investigation* ».

In summary, patients with mild-to-moderate Alzheimer's disease received masitinib as an adjunct to cholinesterase inhibitor and/or memantine treatment for 24 weeks. Improvement in cognitive function and functional capacity was seen in the masitinib treatment group when compared to a placebo group, as evident through the sustained and statistically significant response in ADAS-Cog, as well as the mean change in ADAS-Cog, MMSE, and ADCS-ADL values relative to baseline. These findings were additionally supported by favorable response in the CIBIC-plus and CDR analyses. Such broad benefits are desirable in Alzheimer's disease as this effectively translates into an improved quality-of-life.

Professor Olivier Hermine, President of the scientific committee of AB Science and co-corresponding author on this paper declared: « *Given masitinib's selective targeting of mast cells and specific kinases, the results from this study help establish the role of mast cells in the pathogenesis of neurodegenerative diseases such as Alzheimer's disease. Masitinib may therefore represent an innovative approach in the treatment of*

*Alzheimer's disease. As such, AB Science has launched a clinical development program in the treatment of Alzheimer's disease and is actively preparing to launch a phase 3 study. For this we have already received a positive scientific opinion from the European Medicines Agency (EMA) on the phase 3 study design, which is an important step for the development of masitinib in Alzheimer's disease ».*

### **Summary of the phase 3 study design validated by EMA**

A prospective, multicentre, randomized, controlled, double-blind, parallel-group, phase 3 study to compare the efficacy and safety of masitinib at 6 mg/kg/day versus placebo in the treatment of patients with mild to moderate Alzheimer's disease over a 24 week duration. A total of 300 patients will be enrolled with masitinib being administered orally as an add-on therapy to standard care.

The measure of response will be based upon the proportion of patients presenting:

- An effect on the rate of cognitive decline and memory as evaluated by the Alzheimer's Disease Assessment Scale (ADAS-Cog) at week 24;
- An effect on the functional autonomy and quality-of-life evaluated by the Alzheimer's Disease Cooperative Study Activities of Daily Living (ADCS-ADL) at week 24.

### **About masitinib**

Masitinib is a new orally administered tyrosine kinase inhibitor that targets mast cells, important cells for immunity, as well as a limited number of kinases that play key roles in various cancers. Owing to its novel 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. Through its activity of inhibiting certain kinases that are essential in some oncogenic processes, masitinib may have an effect on tumor regression, alone or in combination with chemotherapy. Through its activity on the mast cell and certain kinases essential to the activation of the inflammatory cells and fibrosing tissue remodeling, masitinib can have an effect on the symptoms associated with some inflammatory and central nervous system 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 new class of targeted molecules whose action is to modify signaling pathways within cells. Through these PKIs, the Company targets diseases with high unmet medical needs (cancer, inflammatory diseases and central nervous system diseases), in both human and veterinary medicines. Thanks to its extensive research and development capabilities, AB Science has its own portfolio of molecules. Masitinib, a lead compound, has already been registered in veterinary medicine in Europe and in the USA, and is pursuing nine phase 3 studies in human medicine, including five studies on-going in pancreatic cancer, GIST, in metastatic melanoma expressing JM mutation of c-Kit, in mastocytosis, and severe persistent asthma.

Further information is available on AB Science's website: [www.ab-science.com](http://www.ab-science.com)

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