



**NEW RESEARCH SHOWS THAT MASITINIB LIMITS NEURONAL DAMAGE IN A MODEL OF NEUROIMMUNE-DRIVEN NEURODEGENERATIVE DISEASE**

**THIS IS THE FIRST DEMONSTRATION THAT MASITINIB CAN LOWER SERUM NEUROFILAMENT LIGHT CHAIN, AN IMPORTANT BIOMARKER FOR NEURODEGENERATIVE DISORDERS**

*Paris, March 13, 2024, 12.45pm CET*

**AB Science SA** (Euronext - FR0010557264 - AB) today announced the publication of new preclinical results for masitinib in neurodegenerative diseases (NDD). Findings have been published on the bioRxiv preprint service as an article entitled, 'Masitinib limits neuronal damage, as measured by serum neurofilament light chain concentration, in a model of neuroimmune-driven neurodegenerative disease'. This article is freely accessible online from the bioRxiv site [1].

The neuroprotective action of masitinib was studied in an animal model of experimental autoimmune encephalitis (EAE). EAE is a model of neuroimmune-driven chronic neuroinflammation and importantly, neuronal damage, or prevention thereof, can be rapidly assessed by measuring serum neurofilament light chain (NfL) concentration in EAE-induced mice. Results showed that masitinib can significantly lower serum NfL levels, and by extension therefore, neuronal damage, in a neuroimmune-driven neurodegenerative disease model, with concomitant reduction in pro-inflammatory cytokines and slowing of clinical symptoms.

Patrick Vermersch, MD, Professor of Neurology at the University of Lille, France, and co-author of this article commented: *"Using a model that is highly relevant to masitinib's mechanism of action in neurodegenerative diseases, this research has shown for the first time that masitinib can lower serum NfL levels, as well as pro-inflammatory cytokines, and by extension therefore, reduce the rate of neuronal damage. Because chronic neuroinflammation is a common pathological characteristic of most neurodegenerative diseases, the observed NfL treatment response indicates that masitinib has a plausible disease-modifying activity in diseases such as progressive multiple sclerosis, amyotrophic lateral sclerosis (ALS) and Alzheimer's disease."*

Alain Moussy, co-founder and CEO of AB Science said: *"The ability of masitinib to impact NFL in an animal model might be important because biomarkers are at the forefront not only of research in neurodegenerative disorders to predict clinical outcome but also regulatory guidelines to accelerate registration. FDA has recognized recently NFL from patients as a potential biomarker for ALS registration and has issued a recent guideline in Alzheimer's Disease encouraging the use of biomarker for accelerated approval. AB Science is not only working on disease biomarkers such as NFL but also on mechanistic biomarkers of the response of masitinib."*

➤ Key points from this research article include:

- Masitinib treatment significantly limited NfL production in EAE mice with respect to the control group, at various timepoints during the 15-day treatment period and in a dose dependent manner.
- Masitinib significantly lowered several well-established pro-inflammatory cytokine biomarker concentrations in EAE mice.
- A beneficial effect of masitinib on functional performance was also observed, with significantly less relative deterioration in grip strength as compared with the control group.

- The measurement of NfL in biological fluids has been proposed for monitoring the therapeutic effect of drugs aimed at reducing axonal damage in various NDDs, including amyotrophic lateral sclerosis, multiple sclerosis, and Alzheimer's disease.
- EAE is a model of neuroimmune-driven chronic neuroinflammation and therefore highly relevant to masitinib's mechanism of action in NDDs.
- Data was derived after disease onset (i.e., in a therapeutic setting as opposed to an asymptomatic preventative setting), which is of greater relevance because such models more closely simulate the clinical condition of NDD patients and therefore better represent their therapeutic needs.

[1] Hermine O, Vermersch P, et al. Masitinib limits neuronal damage, as measured by serum neurofilament light chain concentration, in a model of neuroimmune-driven neurodegenerative disease. Preprint. bioRxiv 2024.03.07.583695; doi: <https://doi.org/10.1101/2024.03.07.583695>  
<https://www.biorxiv.org/content/10.1101/2024.03.07.583695v1.full.pdf+html>

### **About the neurofilament light chain (NfL) biomarker**

The measurement of neurofilament light chain (NfL) in biological fluids has been proposed for monitoring the therapeutic effect of drugs aimed at reducing axonal damage. NfL are cytoskeletal proteins that are highly specific for neurons in both the central nervous system (CNS) and the peripheral nervous system. NfL in cerebrospinal fluid or the bloodstream is therefore indicative of axonal lesions and/or degeneration and elevated NfL levels are associated with traumatic brain injuries or neurodegenerative diseases (NDD), including amyotrophic lateral sclerosis, multiple sclerosis, and Alzheimer's disease. A growing body of literature shows that because the level of free NfL in serum/plasma directly reflects neuronal damage within the CNS, it can be used as a reliable and easily accessible marker of disease intensity and/or activity across a variety of neurological disorders.

### **About bioRxiv**

bioRxiv (pronounced "bio-archive") is a free online archive and distribution service for unpublished preprints in the life sciences. It is operated by Cold Spring Harbor Laboratory, a not-for-profit research and educational institution. By posting preprints on bioRxiv, authors are able to make their findings immediately available to the scientific community and receive feedback on draft manuscripts before they are submitted to journals.

### **About masitinib**

Masitinib is a 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.

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](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 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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