

ExonHit announces publication of EHT 0202 Phase IIa results in Current Alzheimer Research

- Data show good safety and tolerability in patients
- Efforts are ongoing to pursue EHT 0202 development in Phase IIb either through partnering or alternative financing

Paris, France – April 4, 2011 - ExonHit Therapeutics (Alternext: ALEHT) today announced the publication of Phase IIa results for EHT 0202, its lead candidate for the treatment of Alzheimer's disease, in the journal *Current Alzheimer Research*. Study data demonstrate good safety and tolerability of EHT 0202 hence supporting its advancement into Phase IIb to assess clinical efficacy and confirm tolerability in a larger cohort of Alzheimer's patients over a longer treatment duration (1).

"The publication of these promising first patient data in a peer-reviewed Alzheimer's disease journal highlights the interest of the Alzheimer's scientific community in our EHT 0202 compound. Such interest further supports our efforts to pursue the development of EHT 0202 in Phase IIb either through partnering or alternative financing," said Matthew Pando, PhD, Executive Vice President, Therapeutics of ExonHit Therapeutics. *"We have also identified several potential biomarkers, including response signatures using our GWSA platform that will strengthen the likelihood of success in future EHT 0202 clinical trials."*

Study details

The study was conducted in 23 centers across France under the supervision of Professor Bruno Vellas, Head of the Alzheimer's Disease Clinical Research Center and of the Gérontopôle, Toulouse University Hospital, France. A total of 197 ambulatory patients 60 to 90 years old and suffering from mild to moderate Alzheimer's disease were selected and 159 of them were randomized to receive oral study treatment over a three-month period.

This randomized, double-blind, placebo-controlled study was designed to assess the clinical safety and tolerability, as a primary objective, and also exploratory efficacy of EHT 0202 in patients with Alzheimer's disease. The effect of two different doses of EHT 0202 (either 40 or 80 mg twice a day) as adjunctive therapy to one acetylcholinesterase inhibitor was evaluated in comparison to placebo.

About EHT 0202

EHT 0202 is ExonHit's lead candidate in Alzheimer's disease and potentially first in a new class of disease modifying therapies which stimulate the α -secretase pathway, thus enhancing the production of the procognitive and neuroprotective sAPP α fragment of APP (Amyloid Precursor Protein). Since the stimulation of the α -secretase pathway is to the detriment of A β amyloid peptide production, EHT 0202 potentially reduces toxic A β plaque formation (2).

Phase I studies demonstrated good tolerability of EHT 0202 in both young and aged healthy volunteers.

Preclinical studies have shown that EHT 0202 protects cortical neurons against A β 42-induced toxicity and that this neuroprotection is associated with sAPP α induction. EHT 0202 has also demonstrated procognitive properties in several animal models: age-related memory impairment and scopolamine-induced amnesia (3).

About Alzheimer's disease

Alzheimer's disease is a progressive neurodegenerative condition that is the most frequent cause of dementia in the elderly. It is a disease of multifactorial origin that involves environmental and genetic factors. Worldwide, an estimated 26.6 million people had Alzheimer's disease in 2006. This number is set to quadruple by 2050 to more than 100 million; 1 in 85 people worldwide will be living with the disease (4). In France alone, 800,000 people, or 18% of the population over-75, have Alzheimer's disease (5).

About ExonHit Therapeutics

ExonHit Therapeutics (Alternext: ALEHT) is a fast emerging healthcare player active in both therapeutics and diagnostics. The Company is applying its proprietary technology, based on the analysis of alternative RNA splicing, to develop innovative molecular diagnostic tests and therapeutics for Alzheimer's disease and cancer indications. ExonHit has a balanced investment strategy with internal development programs and strategic collaborations, in particular with Allergan and bioMérieux.

ExonHit is headquartered in Paris, France and has U.S. offices in Gaithersburg, Maryland. The Company is listed on Alternext of NYSE Euronext Paris and is part of the NYSE Alternext OSEO innovation index. For more information, please visit <http://www.exonhit.com>.

Disclaimer

This press release contains elements that are not historical facts including, without limitation, certain statements on future expectations and other forward-looking statements. Such statements are based on management's current views and assumptions and involve known and unknown risks and uncertainties that could cause actual results, performance or events to differ materially from those anticipated.

In addition, ExonHit Therapeutics, its shareholders, and its affiliates, directors, officers, advisors and employees have not verified the accuracy of, and make no representations or warranties in relation to, statistical data or predictions contained in this press release that were taken or derived from third party sources or industry publications, and such statistical data and predictions are used in this press release for information purposes only.

Finally, this press release may be drafted in the French and English languages. In an event of differences between the texts, the French language version shall prevail.

References

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