

## ExonHit announces last patient out for EHT 0202 Phase IIa study in Alzheimer

- Top-line EHT 0202 data to be presented in Florence on September 14th

**Paris, France – June 16, 2009** – ExonHit Therapeutics (Alternext: ALEHT) today announced that clinical testing of EHT 0202, its lead therapeutic compound in Alzheimer's disease, is progressing well. Final patient dosing for the Phase IIa proof-of-concept clinical trial assessing EHT 0202 in patients with Alzheimer's disease is completed.

"In light of the neuroprotective and procognitive effects demonstrated by EHT 0202 in preclinical studies, we believe that our drug candidate has the potential to modify the course of Alzheimer's disease and might open a new era in the treatment of this devastating disease," commented Dr. Loïc Maurel, President of the Management Board of ExonHit Therapeutics. "We look forward to the outcome of this Phase IIa trial and are planning to initiate out-licensing discussions with potential partners next fall."

Top-line data from the EHT 0202/002 Phase IIa study will be presented at the 13<sup>th</sup> Congress of the European Federation of Neurological Societies on September 14th in Florence, Italy.

The study was conducted in 23 centers across France under the supervision of Professor Bruno Vellas, Head of Alzheimer's Disease Clinical Research Center and Gerontopole, Toulouse University Hospital, France. A total of 197 ambulatory patients suffering from mild to moderate Alzheimer's disease were selected and 158 of them were randomized to receive oral study treatment over a three-month period.

This multicenter, randomized, double-blind, placebo-controlled study was designed to assess the 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 is evaluated in comparison to placebo. Efficacy is evaluated on multiple parameters by using different scales, including a battery of cognitive assessment tests (ADAS-Cog, NTB, MMSE), assessment of patients' daily living activities, and also global behavioural assessment.

### About EHT 0202

EHT 0202 has a novel mechanism of action when compared to existing Alzheimer's disease therapeutics: it stimulates the  $\alpha$ -secretase pathway, thus enhancing the production of the procognitive and neuroprotective sAPP $\alpha$  fragment of APP (Amyloid Precursor Protein). The stimulation of the  $\alpha$ -secretase pathway being to the detriment of A $\beta$  amyloid peptide production, EHT 0202 potentially reduces toxic A $\beta$  plaque formation (1).

Phase I studies demonstrated good tolerability of EHT 0202 in both young and aged healthy volunteers; importantly, no sedation was observed clinically.

Preclinical studies have shown that EHT 0202 protects cortical neurons against A $\beta$ 42-induced stress and that this neuroprotection is associated with sAPP $\alpha$  induction. EHT 0202 has also demonstrated pro-cognitive properties in several animal models: age-related memory impairment and scopolamine-induced amnesia (2).

## **About Alzheimer's disease**

Alzheimer's disease is a progressive neurodegenerative condition that is the most frequent cause of dementia in the aging population. An estimated 26.6 million people worldwide had Alzheimer in 2006. This number is anticipated to quadruple by 2050 to more than 100 million; 1 in 85 persons worldwide will be living with the disease (3). In France alone, 800,000 people, or 18% of people above 75 years old, have Alzheimer's disease (4).

## **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 blood based diagnostic tests and therapeutics for neurodegenerative and cancer indications. ExonHit has a balanced investment strategy with internal development programs and strategic collaborations, in particular with bioMérieux and Allergan.

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

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*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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