

**EXONHIT THERAPEUTICS REPORTS ADVANCEMENTS IN THE CLINICAL DEVELOPMENT OF EHT 0202, ITS PHASE II DRUG FOR ALZHEIMER'S DISEASE**

- Successful completion of patient enrolment for Phase IIa study
- Study results available in Q4 2009
- Preclinical data suggest EHT 0202 could modify disease evolution

**Paris, France – February 26, 2009** – ExonHit Therapeutics (Alternext: ALEHT) is pleased to announce that clinical testing of EHT 0202, its lead compound in Alzheimer's disease, is progressing well. Patient enrolment for the Phase IIa trial assessing EHT 0202 in patients with Alzheimer's disease is completed.

*"The successful completion of patient enrolment for the Phase IIa represents an important milestone in the development of EHT 0202, and provides a clear timeline for the release of results,"* stated Dr. Loïc Maurel, President of the Management Board of ExonHit Therapeutics. *"Our strategy is to bring EHT 0202 up to the end of the ongoing study and then to look for a partner to move EHT 0202 through further clinical development. We have already met with several pharmaceutical companies that are looking forward to the Phase IIa results."*

The trial is conducted under the supervision of Professor Bruno Vellas, Head of Alzheimer's Disease Clinical Research Center and Gerontopole, Toulouse University Hospital, France.

*"EHT 0202 has an original mechanism of action: it stimulates the  $\alpha$ -secretase pathway. If the neuroprotective and symptomatic effects of EHT 0202 demonstrated in animal models are confirmed in humans, it could change the treatment paradigm for Alzheimer's disease,"* stated Professor Vellas. *"Current available drugs are symptomatic only and their clinical efficacy is limited over time, after which the evolution of the degenerative process starts progressing again. EHT 0202 is an interesting approach to potentially slow the evolution of the disease."*

The Phase IIa trial is a multicenter, randomized, double-blind, placebo-controlled study primarily investigating the safety and tolerability of EHT 0202 in approximately 150 patients with Alzheimer's disease. The effect of two different doses of EHT 0202 as adjunctive therapy to an acetylcholinesterase inhibitor will be evaluated in comparison to placebo. Ambulatory patients suffering from mild to moderate Alzheimer's disease are randomized and receive oral treatment, twice a day, of either 40 or 80 mg of EHT 0202, or placebo over a three-month period. The study design will also allow for the collection of preliminary data related to many clinical efficacy parameters of EHT 0202, notably including a battery of cognitive assessments (ADAS-Cog, NTB, MMSE) but also assessment of patients' daily living activities, global assessment and behaviour.

Study results will be available in Q4 2009.

### **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 or emesis were 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 the most frequent cause of dementia in the aging population. The World Health Organization estimated in 2001 that 18 million people around the world were suffering from Alzheimer's disease and that this figure could nearly double by 2025 to 34 million [3].

### **About ExonHit Therapeutics**

ExonHit Therapeutics is the world's leader in the analysis of alternative RNA splicing, a process which when deregulated plays a key role in the onset of various diseases.

ExonHit Therapeutics has a multi-component commercial strategy to capture the maximum value from its leadership in alternative splicing. The Company is already generating revenues from a new generation of microarrays, SpliceArray™ family of products that enable life science researchers to detect crucial disease-associated information. These products are marketed worldwide in conjunction with Agilent and Affymetrix. In the field of diagnostics, ExonHit Therapeutics has a major collaboration with bioMérieux to develop completely novel predictive blood-based cancer diagnostics, which could play a key role in improving the treatment of breast cancer and other major cancers.

In parallel, ExonHit Therapeutics is developing its own therapeutic pipeline in the field of neurodegenerative diseases and cancer. The Company has advanced drug candidates into clinical trials and is evaluating several promising preclinical compounds. ExonHit Therapeutics also has a strategic partnership with Allergan, to discover and develop new therapeutics in the areas of pain, neurological diseases and ophthalmology. This collaboration provides ongoing research funding to ExonHit.

Founded in 1997, ExonHit is headquartered in Paris, France and has a U.S. facility in Gaithersburg, Maryland. The Company is listed on Alternext of Euronext Paris (ISIN: FR0004054427; ticker: ALEHT) since November 17, 2005. 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

[1] Marcade M, Bourdin J, Loiseau N, Peillon H, Rayer A, Drouin D, Schweighoffer F, Desire L. Etazolate, a neuroprotective drug linking GABAA receptor pharmacology to amyloid precursor protein processing. *Journal of Neurochemistry*. 2008; 106: 392-404

[2] Pando M, Marcade M, Peillon H, Rayer A, Drouin D, Desire L. An alpha-secretase stimulator drug for cognitive disorders associated with neurodegeneration. Presented at the 12<sup>th</sup> congress of the European Federation of Neurological Societies; 23-26 August, 2008; Madrid, Spain

[3] WHO 2001. Alzheimer's disease: The Brain Killer.

WHO website: [http://www.searo.who.int/en/Section1174/Section1199/Section1567/Section1823\\_8066.htm](http://www.searo.who.int/en/Section1174/Section1199/Section1567/Section1823_8066.htm)

## ExonHit Therapeutics

### Media Contact

Corinne Hoff

+33 1 58 05 47 04

corinne.hoff@exonhit.com

### Investor Contact

Philippe Rousseau, CFO

+1 240 404 0191

philippe.rousseau@exonhit.com