PRESS RELEASE

Embargoed until 14 September 2009, 11:30 am CET

ExonHit presents promising first patient results for EHT 0202, its Alzheimer's candidate drug

- Primary endpoints: Good safety and tolerability data supports moving into Phase IIb
- Secondary exploratory endpoints: A trend for efficacy on cognition was observed on ADAS-Cog scores and in ApoE4 positive population
- ExonHit seeking a partner for further development & commercialization of EHT 0202

Paris, France – September 14, 2009 – Top-line Phase IIa clinical data released today demonstrate that EHT 0202, ExonHit's lead candidate for the treatment of Alzheimer's disease, is safe and generally well tolerated in patients and that it could potentially enhance cognition in Alzheimer's disease patients. These first EHT 0202 results in patients were presented today in Florence, Italy, at the 13th Congress of the European Federation of Neurological Societies (1). Further study details will be disclosed at the 2nd Conference of Clinical Trials on Alzheimer Disease in Las Vegas, at the end of October.

"EHT 0202 has an original mechanism of action with potential disease-modifying and symptomatic properties. The Phase IIa safety results and the potential efficacy signals observed in Alzheimer patients support advancing EHT 0202 into later stage clinical development to collect further evidence of safety and efficacy in a larger number of patients and over a longer time period," stated Professor Bruno Vellas, M.D., the principal investigator. *"There is a high unmet need for new Alzheimer treatments with procognitive and neuroprotective properties."*

"*We are very happy about these promising findings as EHT 0202 could become a new approach to the treatment of Alzheimer's disease,*" commented Dr. Loïc Maurel, M.D., President of the Management Board of ExonHit Therapeutics. "*ExonHit will now actively look for a partner to ensure rapid clinical development and commercialization of EHT 0202*."

Data analysis showed that EHT 0202 is safe at both tested doses (40 and 80 mg twice a day) and generally well tolerated. The most frequent adverse events were mainly related to the central nervous system-acting nature of EHT 0202 and were dose-dependent. There were no specific gastrointestinal, cardiovascular or biological adverse events in the treated groups versus placebo suggesting the absence of clinical interaction of EHT 0202 with acetylcholinesterase inhibitors. Study withdrawals related to adverse events happened mostly during the first 6 weeks of treatment and all were due to different causes.

Encouraging signs of cognitive improvement, as measured by ADAS-Cog, the gold standard test for cognition, were seen in EHT 0202 treated patients. These results build on earlier observations using EHT 0202 in animal models of Alzheimer's disease. It was also observed in some assessments including ADAS-Cog that the ApoE4 positive subpopulation (patients with one or two of the ApoE4 alleles in their genes) tends to respond better to EHT 0202 treatment than patients with no ApoE4 allele in their genes.

These first patient data support progressing EHT 0202 into Phase IIb in order to (i) establish EHT 0202's cognitive benefits in a longer and larger clinical trial; (ii) further explore EHT 0202's benefit in the ApoE4 positive population; and (iii) identify the optimal therapeutic dosage for EHT 0202.

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 60-90 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 has a novel mechanism of action when compared to existing Alzheimer's disease therapeutics: it stimulates the α-secretase pathway, thus enhancing the production of the procognitive and neuroprotective sAPPα fragment of APP (Amyloid Precursor Protein). Since the stimulation of the α-secretase pathway is to the detriment of Aβ amyloid peptide production, EHT 0202 potentially reduces toxic Aβ 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 Aβ42-induced toxicity and that this neuroprotection is associated with sAPPα 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 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 (4). In France alone, 800,000 people, or 18% of people above 75 years old, have Alzheimer's disease (5).

Conference call details

The Company together with Professor Vellas, the principal investigator, will be hosting a conference call to discuss these results today, September 14, at 4:00 pm CET. Investors, journalists and financial analysts are invited to participate. Dial-in details and a slideshow are available on ExonHit's website (www.exonhit.com).

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 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.

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) Vellas B et al. A 3-month, randomized, double-blind, placebo-controlled, phase IIa study to assess safety and exploratory efficacy of EHT 0202 as adjunctive therapy in mild to moderate AD patients. Presented at the 13th Congress of the European Federation of Neurological Societies; 12-15 September 2009 Florence, Italy
(2) 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
(3) 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 12th congress of the European Federation of Neurological Societies; 23-26 August, 2008; Madrid, Spain
(4) Brookmeyer R, Johnson E, Ziegler-Graham K, MH Arrighi (July 2007). "Forecasting the global burden of Alzheimer's disease". Alzheimer's and Dementia 3 (3): 186–91
(5) Plan Maladie d'Alzheimer 2004-2007- Ministère des solidarités, de la santé et de la famille

ExonHit Therapeutics

Media Contact
Corinne Hoff
+33 1 58 05 47 04
corinne.hoff@exonhit.com

Investor Contact
Loïc Maurel
+33 1 53 94 77 00
loic.maurel@exonhit.com

ALIZE RP
Caroline Carmagnol
+ 33 6 64 18 99 59
caroline@alizerp.com

Business Development Contact
William Vickery
+33 1 58 05 47 03
william.vickery@exonhit.com