

ExonHit presents new clinical advances on its Alzheimer's diagnostic and therapeutic programs

- Launch of AclarusDx[™] (formerly EHT Dx21) on track for the end of 2009
- Discussions ongoing to out-license EHT 0202

Paris, France – October 30, 2009 – ExonHit Therapeutics (Alternext: ALEHT) yesterday presented clinical data on the validation set "Alzheimer patients versus healthy controls" for AclarusDx[™] (formerly EHT Dx21), its blood-based diagnostic test for the detection of Alzheimer's disease (AD), at the 2nd Conference of Clinical Trials on Alzheimer's Disease (CTAD) in Las Vegas, USA. This set of data will support the launch of a test designed to differentiate AD patients from healthy individuals and will be the first assay of the AclarusDx[™] product line.

"Many challenges exist today with respect to the clinical diagnosis of AD patients," stated Peter J. Snyder, M.D., Ph.D., Vice President for Research for Lifespan and a Professor of Clinical Neurosciences at the Warren Alpert Medical School of Brown University, Providence, Rhode Island. "Moreover, there is a strong unmet need on the part of pharmaceutical companies to utilize reliable, valid and cost-effective biomarkers that could improve patient selection for AD clinical trials. AclarusDxTM may be an important new tool to help identify clinical populations who will potentially benefit most from novel and exciting therapeutic advances."

"With the availability of AclarusDx[™], ExonHit will become one of the first companies in the world to offer a blood diagnostic test devoted to Alzheimer's disease. The use of such assay, simple to perform, will be a tremendous step forward compared to the current qualitative diagnostic tests," commented Loïc Maurel, M.D., President of the Management Board of ExonHit Therapeutics. "We have a fully operational and GLP compliant laboratory in our US facility capable of supporting AD studies for the pharmaceutical industry."

The patient population for the clinical validation study covered the range of severe through mild AD to ensure the signature performed across the continuum of the disease state where subjects presenting symptoms of Alzheimer's disease were having a MMSE (Mini-Mental State Examination) score of less than 28. Over 200 individuals were assessed in the study. From this total (110 patients with AD and 101 control subjects), AclarusDx[™] showed an overall accuracy of 71% identifying patients with Azheimer's Disease correctly in 74% of all cases. The identification and removal of ambiguous samples (approximately 25%) increased the accuracy of the test to 75%, and the number of correctly identified AD patients to 80% (1). Scientific findings published recently in *Lancet Neurology* indicate that over 30% of healthy controls have profiles suggestive of AD based on analysis of their cerebral spinal fluid, obtained by doing a lumbar puncture (2). The results from the clinical validation study suggest that AclarusDx[™], a non-invasive blood based test, can be used within the battery of cognitive instruments to help refine and classify the patient population for clinical trials.

ExonHit will first introduce AclarusDx[™] as a Research Use Only service to pharmaceutical companies and leading academic centers conducting clinical trials in Alzheimer's disease. The clinical diagnostics market will be served through working with partners, following CE marking in Europe and In Vitro Diagnostic registration in the USA.

For EHT 0202, ExonHit's lead therapeutic candidate in Alzheimer's disease, additional Phase IIa data will be presented later today also at the CTAD (3). These results further support the initial findings presented on September 14 at the 13th Congress of the European Federation of Neurological Societies in Florence, Italy (4). They confirm that EHT 0202 is safe and generally well tolerated in patients and demonstrated an excellent compliance rate (>95%) for the study. They also show that overall blood levels of EHT 0202 and its main metabolites were stable during the study, suggesting that no change in the molecule's safety profile related to plasma levels is to be expected during longer administration periods. Also, the positive efficacy trend in the ApoE4 positive subpopulation was confirmed on different assessment parameters. However due to the limited size and duration of the study, no statistically significant changes were observed in the other efficacy scales (NTB, CDR-SB, Zarit, NPI, CGI).

These first patient data support the advancement of EHT 0202 into Phase IIb and ExonHit is in discussion with potential partners for further development and commercialization of EHT 0202.

About AclarusDx[™] (formerly EHT Dx21)

ExonHit Therapeutics has an extensively validated technical platform to analyze transcript variations in a standardized, GLP compliant environment with demonstrated reproducibility. This validated platform has been used in a large clinical study to demonstrate its ability to select AD patients through a simple blood sample and hence led to the development of the AclarusDx[™] Alzheimer product. The information generated by the assay can be expanded and applied to clinical research applications such as pharmagenomics.

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 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 (5).

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 (6).

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 (7). In France alone, 800,000 people, or 18% of people above 75 years old, have Alzheimer's disease (8).

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.

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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) Fehlbaum-Beurdeley P et al. Identification of patients with Alzheimer's disease using molecular signatures derived from splice variant expression profiles from peripheral blood. Presented at the 2nd Conference of Clinical Trials on Alzheimer's Disease in Las Vegas, on October 29, 2009.

(2) Visser P. et al. Alzheimer's disease pathology in patients with subjective cognitive impairment or mild cognitive impairment in the DESCRIPA study: a prospective cohort study. Lancet Neurology 2009; 8: 619–27.
(3) Vellas B, Ousset PJ, Sol O, Desire L, Pando M. Safety and exploratory efficacy of EHT 0202 in mild to moderate AD patients: a 3-month, randomized, double-blind, placebo-controlled, phase IIa study. Presented at the 2nd Conference of Clinical Trials on Alzheimer's Disease in Las Vegas, on October 30, 2009.

(4) 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

(5) 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

(6) 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

(7) 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

(8) Plan Maladie d'Alzheimer 2004-2007- Ministère des solidarités, de la santé et de la famille

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