Monitoring patients genomic expression in clinical trials for Alzheimer's Disease (AD) can assist trial design and treatment response analysis. EHT 0202 is a new compound with potential disease-modifying and symptomatic properties for AD. Here, using Exonhit's SpliceArray™ technology, we report on the identification in AD patients of blood-based transcriptomic signatures associated with treatment response of EHT0202 in a 3-month phase IIA study aimed at determining the clinical safety, tolerability and exploratory efficacy of EHT0202 (40 and 80mg bid) as adjunctive therapy to one cholinesterase inhibitor in mild to moderate AD patients. A research endpoint was to identify prospective blood expression biomarkers for predicting patient response to EHT0202 on cognitive impairment.

Sixty AD patients (20 in each study groups) were selected that either improved or declined during the study with regards to ADAS-cog total score. Analyses were performed to identify predictive and efficacy biomarkers from blood transcriptome using the SpliceArray™ technology in relation to treatment response of EHT0202. Biomarkers were identified to associate with response and correlated with BMI, a known risk factor for dementia. In addition, the involved genes play significant roles in metabolic activity, supporting the biological implication of the biomarkers. Efficacy biomarkers were also identified and will be presented. These biomarkers will need to be validated on larger cohorts.

In conclusion, pharmacogenomic analyses including the SpliceArray™ technology can be applied in clinical trials to discover biomarkers for prospective identification of patients who can benefit from a treatment but also follow their response to that treatment.