

## ExonHit launches first product in Alzheimer

- AclarusDx™ Alzheimer's test now available as a Research Use Only product
- Strong need for accurate patient characterization in Alzheimer clinical trials
- Clinical guidance from a world-class Alzheimer Scientific Advisory Board

**Paris, France – December 3, 2009** – ExonHit Therapeutics (Alternext: ALEHT) is pleased to announce that AclarusDx™ Alzheimer's test (formerly known as EHT Dx21), its blood-based test for the detection of Alzheimer's disease (AD), is now available as a Research Use Only (RUO) product for pharmaceutical companies and leading academic centers conducting clinical trials in Alzheimer's disease.

*"Alzheimer's disease is a multi-factorial and a complex pathology. Currently, its diagnosis is mainly based on clinical criteria, i.e., relies on subjective measurements. Including subjects who are not properly characterized may easily increase the cost of a clinical study and potentially jeopardize its success,"* commented Loïc Maurel, M.D., President of the Management Board of ExonHit Therapeutics. *"The use of AclarusDx™ Alzheimer's test could be a tremendous asset for the pharmaceutical industry as it has the potential to identify populations of patients who might benefit the most from therapeutic advances in AD."*

AclarusDx™ Alzheimer's test is a reproducible, objective, and non invasive test. A blood sample is all that is required from the patient, so it can be implemented at any recruiting site. Unlike brain imaging techniques or cerebrospinal fluid sampling, no special equipment or medical facilities are required and the ease of the procedure should enhance volunteers' willingness to participate in trials. It may also increase the recruitment speed and allow the clinical trial sponsor to include investigating centers other than the main hospitals. When used in combination with neuro-cognitive tests, AclarusDx™ Alzheimer's test could help pharmaceutical companies running clinical trials to include patients fulfilling clinical criteria for AD and who share a common transcriptional profile that has been correlated to AD. Hence, AclarusDx™ Alzheimer's test could lower the trial's "background noise" and potentially lead to a better study outcome by selecting a more homogeneous study population.

In 2008, 43 clinical trials involving approximately 10,700 patients were initiated to test new AD therapies. In 2009, 70 AD clinical studies involving around 14,000 patients were started (1). To recruit one Alzheimer patient, 3 to 4 individuals have to be screened (2).

The launch of AclarusDx™ Alzheimer's test as a RUO product is the first step in ExonHit's commercial strategy in which trial sponsors will send patient blood samples for analysis in ExonHit's GLP-compliant laboratories in the US. In parallel, the Company is preparing the launch of its product in the clinical *in vitro* diagnostics (IVD) market with partners. ExonHit is anticipating a CE marking in Q4 2010 for a European launch in Q1 of 2011. Regarding US marketing approval, discussions have been initiated with the FDA to define the exact regulatory path to meet IVD requirements.

ExonHit Therapeutics has a unique approach to Alzheimer's disease with both therapeutic (EHT 0202, its lead candidate in Phase II) and diagnostic programs (AclarusDx™ Alzheimer's test) in clinical development. For its research and development work in AD diagnostics and more recently therapeutics, ExonHit is counseled by experts from both sides of the Atlantic: Jeffrey Cummings, M.D., Serge Gauthier, M.D., Zaven Khachaturian, Ph.D., Peter Snyder, Ph.D. and Bruno Vellas, M.D..

### **About ExonHit's Alzheimer Scientific Advisory Board**

The Board advises the Company on the development of novel therapies and the clinical development of molecular diagnostics for the research community, the pharmaceutical industry and the healthcare community at large with a particular emphasis on strategies for disease prevention.

*Jeffrey Cummings, M.D.*, is the Augustus Rose Professor of Neurology, Director of the Mary S. Easton Center for Alzheimer's Disease Research, and Director of the Deane F. Johnson Center for Neurotherapeutics at the David Geffen School of Medicine at UCLA (Los Angeles, CA). He is the 2008 recipient of the National Alzheimer's Association's Ronald and Nancy Reagan Research Award. Professor Cummings has authored nearly 1,000 articles, chapters, and abstracts on Alzheimer's disease and other brain diseases.

*Serge Gauthier, M.D.*, is a Professor within the Departments of Psychiatry, Neurology & Neurosurgery, and Medicine at McGill University (Montréal, Canada). He is also Director of the Alzheimer's Disease and Related Disorders Unit at the McGill Center for Studies in Aging, Douglas Mental Health University Institute. In 2006, he received the "Prix Ambassadeur" from the Montréal Palais des Congrès and in 2007 was elected as one of the Best Doctors in North America®.

*Zaven Khachaturian, Ph.D.*, is a Senior Associate of Khachaturian and Associates (Potomac, MD). He was formerly President and CEO of the Lou Ruvo Brain Institute (Las Vegas, NV) which is dedicated to finding cures for memory, mood and movement disorders associated with dementia diseases (including Alzheimer's). Also, for nearly two decades, Dr. Khachaturian led the Alzheimer's disease research department at the U.S National Institutes of Health. In 1984, Dr. Khachaturian posited a unifying theory of brain aging, which is now referred to as the "Calcium Hypothesis of Brain Aging". Zaven Khachaturian is a former member of the FDA's Peripheral and Central Nervous System Advisory Committee and remains as *ad hoc* consultant.

*Peter Snyder, Ph.D.*, is Vice President for Research for the Lifespan Hospital System (including Rhode Island Hospital) and Professor of Neurology at the Alpert Medical School of Brown University (Providence, RI). Earlier in his career, Dr. Snyder was an Early Clinical Leader and Director at Pfizer Pharmaceutical's Global Research & Development laboratories in Groton (Connecticut, USA). In 2008, he was honored as a Fulton-Cushing Lecturer in the Department of Neurosurgery at the Yale University School of Medicine. Dr. Snyder is the Associate Editor of Brain and Cognition, and he maintains an active research program in Alzheimer's disease.

*Bruno Vellas, M.D.*, is a Professor of Internal Medicine & Geriatrics and heads the Alzheimer's Disease Clinical Research Center and the Gerontopole at Toulouse University Medical Center (Toulouse, France). He is a Principal investigator within the European Alzheimer's Disease Consortium and co-leads the Epidemiology and Sociology of Aging research group at Inserm Unit 558.

### **About Alzheimer's disease**

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

*Note: AclarusDx™ Alzheimer's test is available as a Research Use Only product. It is a research tool and should not be used to diagnose patients in a clinical diagnostic setting until its registration as an IVD product in Europe and in the US*

## **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**

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*Lastly, 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) [www.clinicaltrials.gov](http://www.clinicaltrials.gov)
- (2) CRO Market Surveillance report – IMS Health Global, March 2006 (Pharmaceutical/Biotech Market and Clinical Development)
- (3) 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
- (4) Plan Maladie d'Alzheimer 2004-2007- Ministère des solidarités, de la santé et de la famille

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