

## **ORYZON presents final data on the Phase I trial with ORY-2001 at the Alzheimer's Association International Conference (AAIC-2017)**

- ❖ **Excellent safety data profile on 106 healthy volunteers**
- ❖ **The molecule is brain penetrant**
- ❖ **Data allows establishment of a safe administration scheme for long term efficacy studies**

**BARCELONA, SPAIN and CAMBRIDGE, MA, July 24, 2017** – Oryzon Genomics (ISIN Code: ES0167733015, ORY), a public clinical-stage biopharmaceutical company leveraging epigenetics to develop therapies in diseases with strong unmet medical need, presented last week final data on the First in Human clinical trial of ORY-2001 at the Alzheimer's Association International Conference (AAIC-2017), which took place from 16 to 20 July in London (United Kingdom). Oryzon's communication, on poster session S8, was entitled "*First-in-Human Phase I Results Show Safety, Tolerability and Brain Penetrance of ORY-2001, an Epigenetic Drug Targeting LSD1 and MAO-B*" and was presented by Dr. Tamara Maes, Chief Scientific Officer, and Dr. Roger Bullock, Chief Medical Officer of the company.

ORY-2001, a dual LSD1/MAO-B inhibitor, is a novel epigenetic drug for the treatment of neurodegenerative diseases and the second compound the company has moved into clinical trials. ORY-2001 has provided robust preclinical therapeutic activity, restoring memory and other parameters in SAMP-8 mice, a model for accelerated aging and Alzheimer's disease, as well as in other models. LSD1 is a protein that participates in transcription regulation complexes; its modulation can be used to tweak transcriptional imbalances in neurodegenerative disease and redress neuroinflammation and cognitive deficit.

The Phase I trial, carried out at the Drug Research Center of the Santa Creu I Sant Pau Hospital Research Institute (CIM-Sant Pau), has evaluated the safety, tolerability, pharmacokinetics and pharmacodynamics of ORY-2001. The controlled administration to healthy volunteers of ascending, first single (SAD) and then multiple doses (MAD) of ORY-2001 has successfully completed without significant clinical changes detected. Tests have included a comprehensive panel of laboratory tests, vital signs, electrocardiograms, physical examinations and monitoring of adverse events. With the approval of the Spanish Medicines Agency (AEMPS), an additional cohort of volunteers was added to be administered at a higher dose of ORY-2001 to complete the hematological safety profile of the product, as well as two additional cohorts to determine the levels of the drug in cerebrospinal fluid. A total of 106 volunteers, young and old, have participated in the study.

The safety data have been fully satisfactory without significant clinical changes. Tolerance observed in the elderly is similar to that of young volunteers. Pharmacokinetic behavior has been linear with different doses and the half-life of the drug allows ORY-2001 to be effectively administered with a single daily oral dose. Brain exposure was determined by measuring drug levels in cerebrospinal fluid at two different doses. The pharmacodynamics of peripheral target engagement of ORY-2001 to LSD1, analyzed

using a patented proprietary assay developed by the company, showed a time and dose dependent profile that can be correlated to the pharmacokinetics data. In summary, this Phase I study has provided detailed information that allows modeling of the dose response in human vs preclinical species and the establishment of a safe administration scheme for long term Phase II efficacy studies of ORY-2001 in patients with neurodegeneration and neuroinflammation. The company aims to file the corresponding CTA/INDs in the second semester of 2017.

Dr. Carlos Buesa, CEO of Oryzon, commented: "The results of this study are very positive and informative. Oryzon is leading the epigenetic approach to the treatment of neurodegenerative diseases and we are firmly committed to trying to help patients afflicted by these terrible diseases with our novel drugs. The company's recent funding last April allows us to move forward rapidly in the next Phases II and we are going to focus very strongly on this execution in the coming months". Dr. Roger Bullock, Chief Medical Officer of Oryzon and a well-known international field specialist, has stated: "This is an exciting moment for Oryzon. We now have shown brain target engagement and know the appropriate doses ready for the next phase II studies. This whole new approach to preserving neuronal function through epigenetic mechanisms is based on cutting edge science and I am very proud to be involved in bringing these important studies into the clinical arena".

#### **About Oryzon**

Founded in 2000 in Barcelona, Spain, Oryzon (ISIN Code: ES0167733015) is a clinical stage biopharmaceutical company considered as the European champion in Epigenetics. The company has one of the strongest portfolios in the field. Oryzon's LSD1 program is currently covered by + 20 patent families and has rendered two compounds in clinical trials. In addition, Oryzon has ongoing programs for developing inhibitors against other epigenetic targets. The company has a strong technological platform for biomarker identification and performs biomarker and target validation for a variety of malignant and neurodegenerative diseases. Oryzon's strategy is to develop first in class compounds against novel epigenetic targets through Phase II clinical trials, at which point it is decided on a case-by-case basis to either keep the development in-house or to partner or outlicense the compound for late stage development and commercialization. The company has offices in Barcelona and Cambridge, Massachusetts. For more information, visit [www.oryzon.com](http://www.oryzon.com).

#### **FORWARD-LOOKING STATEMENTS**

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