# Oryzon Initiates Multiple Ascending Dose Cohorts In ORY-2001 Phase 1 Clinical Trial

BARCELONA, SPAIN and CAMBRIDGE, MA, July 27, 2016 – Oryzon Genomics (ISIN Code: ES0167733015, ORY), a public clinical-stage biopharmaceutical company leveraging epigenetics to develop therapies in diseases with strong unmet medical need, announced today the initiation of multiple ascending dose cohorts in the on-going Phase 1 study of its proprietary oral epigenetic drug ORY-2001 in healthy subjects. This Phase I study is being conducted in Spain.

During an earlier segment of this Phase 1 study, the safety, tolerability, pharmacokinetics and pharmacodynamics of ORY-2001 have been assessed in several cohorts, with a total of 40 healthy volunteers administered with single doses of the drug. Data generated from these single dose cohorts has been satisfactory and enabled this next step. The multiple ascending dose cohorts will involve 48 additional volunteers including elderly subjects.

With a successful study outcome, ORY-2001 is expected to proceed to a Phase II study in Alzheimer's disease patients in 1H 2017. "We are pleased to continue clinical development of ORY-2001, which to our knowledge is the first histone demethylase inhibitor to be tested in humans for CNS conditions," said Dr. Cesar Molinero, Oryzon's Chief Medical Officer.

ORY-2001 is a highly selective dual LSD1-MAOB inhibitor. The molecule, which focuses on cognitive decline and memory loss, has a good safety profile and therapeutic index in preclinical trials. In nontransgenic AD mouse models, long-term treatments with the drug demonstrated a marked cognitive improvement. The company is also exploring its potential in other CNS disorders.

LSD1 is an epigenetic modulator, which regulates histone methylation. Epigenetic approaches to modify the progression of various neurodegenerative diseases focus on producing changes in patterns of gene expression in neurons and also in glia cells and are of interest for the pharmaceutical industry.

Oryzon has a highly competitive and productive Epigenetics Platform, centered around the development of LSD1 inhibitors, with a first drug currently in Phase IIA in acute leukemia that validates the platform scientifically and clinically. This Platform has so far produced two compounds in clinical development with multiple indication opportunities for both drugs. Oryzon's LSD1 drugs are pioneering the Histone demethylases field with its forerunner oncologic drug that was licensed to Roche in 2014 and ORY-2001 in Phase I in neurodegenerative disorders. In addition, Oryzon has nominated recently a third epigenetic compound, also against LSD1, as preclinical candidate for a yet undisclosed non-oncological orphan indication.

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

Alzheimer's disease is the most common form of dementia in adults. It is estimated to affect 5.3 million Americans and over 30 million people worldwide with an average course of 8-12 years. It is projected that the disease prevalence will double over the next 20 years. Marketed products address some of the symptoms, but there are no treatments currently available. The economic cost of Alzheimer's is expected to grow in the coming years. Projections of the direct cost of Alzheimer's disease in adults over 65 could balloon to \$1.1 trillion in 2050 (in today's dollars) with a total of \$20.8 trillion in medical costs between 2015 and 2050, according to the Alzheimer's Association.

#### **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 and a clinical asset already partnered with Roche. 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.

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

This communication contains forward-looking information and statements about Oryzon Genomics, S.A., including financial projections and estimates and their underlying assumptions, statements regarding plans, objectives and expectations with respect to future operations, capital expenditures, synergies, products and services, and statements regarding future performance. Forward-looking statements are statements that are not historical facts and are generally identified by the words "expects", "anticipates", "believes", "intends", "estimates" and similar expressions. Although Oryzon Genomics, S.A. believes that the expectations reflected in such forward-looking statements are reasonable, investors and holders of Oryzon Genomics, S.A. shares are cautioned that forwardlooking information and statements are subject to various risks and uncertainties, many of which are difficult to predict and generally beyond the control of Oryzon Genomics, S.A., that could cause actual results and developments to differ materially from those expressed in, or implied or projected by, the forward-looking information and statements. These risks and uncertainties include those discussed or identified in the documents sent by Oryzon Genomics, S.A. to the Comisión Nacional del Mercado de Valores, which are accessible to the public. Forward-looking statements are not guarantees of future performance. The auditors of Oryzon Genomics, S.A, have not reviewed them. You are cautioned not to place undue reliance on the forward-looking statements, which speak only as of the date they were made. All subsequent oral or written forward-looking statements attributable to Oryzon Genomics, S.A. or any of its members, directors, officers, employees or any persons acting on its behalf are expressly qualified in their entirety by the cautionary statement above. All forward-looking statements included herein are based on information available to Oryzon Genomics, S.A. on the date hereof. Except as required by applicable law, Oryzon Genomics, S.A. does not undertake any obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

US Contact: Spain: The Company:

The Trout Group ATREVIA Ms. Anna K Baran

Maria Lomaka Patricia Cobo IR Director

+1 646 378 2932 +34 91 564 07 25 +44 (0) 752 1083 006

mlomaka@troutgroup.com pcobo@atrevia.com abaran@oryzon.com