

## ORYZON Announces Publication of Paper in *Cancer Cell* establishing the relevance of ORY-1001 as an antileukemic differentiating drug

MADRID, SPAIN and CAMBRIDGE, MA, March 05, 2018 – Oryzon Genomics (ISIN Code: ES0167733015, ORY), a public clinical-stage biopharmaceutical company leveraging epigenetics to develop therapies in diseases with strong unmet medical need, has announced today the publication by company scientists of a new paper in the March issue of *Cancer Cell*. The paper describes the development of ORY-1001, a highly selective KDM1A inhibitor with unrivalled *in vivo* potency. KDM1A (also known as LSD1) is a histone lysine demethylase, reported to be essential for leukemic stem cell maintenance.

The manuscript, titled "*ORY-1001, a potent and selective covalent KDM1A inhibitor, for the treatment of Acute Leukemia*" details that KDM1A pharmacological inhibition with therapeutically-relevant doses of ORY-1001 results in AML differentiation and tumor growth reduction. The *in vitro* and *in vivo* efficacy data presented in the manuscript have been confirmed by measurement of KDM1A target engagement and gene-specific increases of H3K4me2 levels. The mechanism of action of the molecule was further addressed by transcriptomics and chemoproteomics.

"These findings provide a detailed characterization of the pharmacology of ORY-1001, the first LSD1 inhibitor that was moved to human clinical trials and the first to report differentiating activity in AML patients. In addition, the pre-clinical studies revealed important synergy of ORY-1001 with other marketed and experimental anti-leukemia treatments, highlighting the potential of ORY-1001 in combination therapies" said Dr. Tamara Maes, co-founder, VP and CSO of Oryzon.

ORY-1001 has finalized a Phase I-IIA study in refractory or relapsed Acute Leukemia patients. ORY-1001 showed a good safety and tolerability profile, clear evidence of target engagement and mechanism of action, and one CRi. The complete results of this trial will be reported in a separate paper. "The company is preparing two exploratory Phase IIA trials of ORY-1001 in combination with other agents in AML and in SCLC that are expected to start in 2H2018" commented Dr. Roger Bullock, CMO of the company.

The paper can be accessed here: [http://www.cell.com/cancer-cell/fulltext/S1535-6108\(18\)30023-0](http://www.cell.com/cancer-cell/fulltext/S1535-6108(18)30023-0)

### **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 has resulted in + 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 Spain and USA. For more information, visit [www.oryzon.com](http://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 forward-looking 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. This press release is not an offer of securities for sale in the United States. The Company’s securities may not be offered or sold in the United States absent registration or an exemption from registration. Any public offering of the Company’s securities to be made in the United States will be made by means of a prospectus that may be obtained from the Company or the selling security holder, as applicable, that will contain detailed information about the Company and management, as well as financial statements.

**US Contact:**

**The Trout Group**  
**Thomas Hoffmann**  
+1 646 378 2932  
[thoffmann@troutgroup.com](mailto:thoffmann@troutgroup.com)

**Spain:**

**ATREVIA**  
**Patricia Cobo/Luis Rejano**  
+34 91 564 07 25  
[pcobo@atrevia.com](mailto:pcobo@atrevia.com)  
[lrejano@atrevia.com](mailto:lrejano@atrevia.com)

**The Company:**

**Emili Torrell**  
**BD Director**  
+34 93 515 13 13  
[etorrell@oryzon.com](mailto:etorrell@oryzon.com)