

Preclinical Efficacy Data on Sickle Cell Disease of ORY-3001, a LSD1 inhibitor from ORYZON, to be presented at ASH 2017

- **Research in mice and baboons has been done by the University of Illinois at Chicago**
- **Oral Presentation on December 10th to be delivered by Prof. D. Lavelle**
- **Oryzon's CEO will attend the Conference**

MADRID, SPAIN and CAMBRIDGE, MA, December 4th, 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, today announced that preclinical efficacy data of its investigational drug ORY-3001, a selective LSD1 inhibitor, will be presented at an oral presentation at the American Society of Hematology (ASH) 59th Annual Meeting and Exposition, being held December 9-12, 2017 in Atlanta, GA.

Professor Donald Lavelle from the Department of Medicine, University of Illinois at Chicago, Jesse Brown VA Medical Center, Chicago, IL, who has led the collaborative research, will present the communication entitled "Oral Administration of the LSD1 Inhibitor OG-S1335 Increases Fetal Hemoglobin in Humanized Transgenic Sickle Cell Disease Mice and in Baboons".

Sickle cell disease (SCD) is a genetic disease in which the gene for adult hemoglobin is mutated and abnormal shaped red blood cells are produced. Red cells are shaped like a sickle. This leads to anemia. The sickle cells also get stuck in blood vessels, blocking blood flow. This can cause anoxia in the tissues with inflammatory reactions, pain and organ damage. Professor Lavelle will present data confirming that LSD1 inhibition upregulates Fetal hemoglobin (HbF) gene expression which are normal and may replace partially the function of the mutated adult versions, causing a general improvement. Oral administration of ORY-3001 increases 10-fold the HbF in SCD transgenic mice and F retics levels by 300%, which may compensate the anemia. In baboons, F-retics increase is 8-fold. These, and other data, confirm that oral administration of the LSD1 inhibitor ORY-3001 may be a promising therapeutic option, supporting further efforts toward the development of this drug for SCD therapy.

Oryzon has several selective LSD1 inhibitors in clinical or ready to enter into clinic. Oryzon's CEO, Dr. Carlos Buesa, will attend the conference and said: "We are very pleased with the result of this collaboration with Dr. Lavelle and the University of Illinois. These results with ORY-3001 are very positive and promising. Our LSD1 inhibitors are sufficiently selective and pharmacologically refined to consider them as a therapeutic option in addition to their uses in

oncology. The FDA is encouraging the industry to provide new alternatives to treat SCD patients who are underserved."

Sickle-cell disease affected approximately 150,000 people in the US in 2014, and there is no cure but palliative treatments. The median survival estimated in the US in 1994 was 42 for men and 48 for women. The average cost for a pediatric patient is about \$ 15,000 per year. SCD is a major public health concern. From 1989 through 1993, an average of 75,000 hospitalizations due to SCD occurred in the US, costing approximately \$475 million.

A summary of the preliminary preclinical data that will be presented at ASH is available on the official website of the congress:

<https://ash.confex.com/ash/2017/webprogram/Paper99635.html>.

Oral presentation Information

356 Oral Administration of the LSD1 Inhibitor OG-S1335 Increases Fetal Hemoglobin in Humanized Transgenic Sickle Cell Disease Mice and in Baboons

Program: Oral and Poster Abstracts

Type: Oral

Session: 112. Thalassemia and Globin Gene Regulation II

Sunday, December 10, 2017: 9:45 AM

Bldg B, Lvl 2, B213-B214 (Georgia World Congress Center)

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.

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**Spain:****ATREVIA****Patricia Cobo/Luis Rejano**

+34 91 564 07 25

pcobo@atrevia.com**lrejano@atrevia.com****The Company:****Emili Torrell****BD Director**

+34 93 515 13 13

etorrell@oryzon.com