

ORYZON Reports Financial Results and Corporate Update for the 3rd Quarter Ended September 30, 2017

MADRID, SPAIN and CAMBRIDGE, MA, November 7, 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 reported financial results for the third quarter of 2017 and provided an update on the Company's recent developments.

The company is progressing satisfactorily the dialogue with Roche to implement the termination of the license agreement between both companies relating to ORY-1001 signed in 2014. The company expects to regain full control of this asset in mid-January. The company has started an intense dialogue with KOLs and regulatory experts in US and Europe to define the best clinical positioning for ORY-1001 in the onco-hematological cancers and solid tumors.

The Phase I clinical trial of ORY-2001, a dual inhibitor of LSD1 and MAOB, in healthy volunteers to assess its potential in Alzheimer's disease and Multiple Sclerosis was completed with satisfactory results. Final data was presented at the AAIC meeting in London. The drug was safe and well tolerated. Tolerance observed in the elderly was similar to that of young volunteers. Pharmacokinetic behavior was linear with different doses and the half-life of the drug allows single daily oral dosing. Brain exposure was determined by measuring drug levels in cerebrospinal fluid in 18 volunteers using two different doses. Brain penetrance was high and established at 0.7-0.9 fold the unbound plasma levels. 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 provided detailed information allowing 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. Additionally, the company presented new data on preclinical efficacy of ORY-2001 at *MSParis2017*, the joint meeting of ECTRIMS and ACTRIMS, the European and Americas Committees for Treatment and Research in Multiple Sclerosis, held in Paris, France, with a poster entitled "*Characterization of the efficacy of ORY-2001, a novel epigenetic drug for the treatment of multiple sclerosis, during the effector phase of the EAE model*". Among other data, the company reported that in the acute phase of the autoimmune attack in this animal model, side to side experiments showed a deeper and/or faster protection on the animals treated with ORY-2001 than the ones treated with other approved agents. This communication was shortlisted by the Scientific Committee of the Congress to opt for one of the 5 Poster Awards of the Congress.

The Company submitted a Clinical Trial Application (CTA), the European IND equivalent, to the Spanish Drug Agency (AEMPS) to start a Phase IIA trial with ORY-2001 in relapse-remitting and secondary progressive MS patients last July. The company has recently received approval of this CTA. The study, named SATEEN (**SA**fety, **T**olerability and **E**fficacy in an **EPI**GENETIC approach to treat Multiple Sclerosis), will be conducted in different Spanish hospitals, and is designed as a randomised, double-blind, placebo-

controlled, 3-arm, 36 weeks parallel-group study to evaluate the safety and tolerability of ORY-2001 in patients with Relapsing-Remitting Multiple Sclerosis (RRMS) and Secondary Progressive Multiple Sclerosis (SPMS).

The company has continued its experimental work with ORY-2001 in preclinical models of Alzheimer's disease and other CNS indications and has done substantial advances in the characterization of the mechanism of action of ORY-2001 in different CNS diseases. This broadens the therapeutic indication's potential for the Clinical Development Plan of this drug. The company will present these and other data in specialized conferences like the SfN's 47th annual meeting (Neuroscience 2017), the world largest conference in Neuroscience to be held in Washington DC, and others to be announced soon.

ORY-3001, the company's third LSD1 inhibitor, currently in preclinical development for the treatment of a non-oncological, yet undisclosed, orphan disease, continues successfully finalized the regulatory toxicology package.

Third Quarter Highlights

- ORYZON announced it will regain rights to ORY-1001
- ORYZON presents final data on the Phase I trial with ORY-2001 at the Alzheimer's Association International Conference (AAIC-2017)
- In OCTOBER 2017 ORYZON receives approval from AEMPS to start SATEEN: a Phase IIA clinical trial in Multiple Sclerosis with ORY-2001

Financial Update: Third Quarter 2017 Financial Results

Collaboration revenue was \$0.0 and \$0.2 million for the first 3 and 9 months ended September 30, 2017 and \$0.2 and \$0.8 million for the first 3 and 9 months ended September 30, 2016. The 3rd quarter 2017 revenues are the last accrual of the Roche license 2015 milestone.

Research and development (R&D) expenses established themselves at \$1.5 and \$5.1 million for the first 3 and 9 months ended September 30, 2017 compared to the \$1.5 and \$4.0 million for the first 3 and 9 months ended September 30, 2016. The \$1.1 million increase was driven primarily by accelerated R&D efforts in the ORY-2001 program.

General and administrative expenses were \$1.0 and 3.3 million for the first 3 and 9 months ended September 30, 2017 and \$1.4 and \$4.2 million for the first 3 and 9 months ended September 30, 2016. This decrease is primarily due to the fact that during the third quarter of 2016 the company incurred in specific non-recurring expenses related with the activities to list the company in the Spanish stock market.

Net loss for the first 3 and 9 months ended September 30, 2017 was \$1.4 and \$4.6 million (-\$0.04 per share) compared to a net loss of \$1.5 and \$4.4 million for the 3 and 9 months ended September, 2016 (-\$0.05 per share).

Cash, cash equivalents and marketable securities totaled \$40.0 million as of September 30, 2017, compared to \$32.1 million as of September 30, 2016.

ORYZON GENOMICS SA
BALANCE SHEET DATA (UNAUDITED)
(Amounts in thousands US \$)

	<u>September 30th, 2017</u>	<u>September 30th, 2016</u>
Cash and cash equivalents	39.841	25.900
Marketable securities	200	6.248
Total Assets	<u>69.741</u>	<u>56.564</u>
Deferred revenue	<u>0</u>	<u>0</u>
Total Stockholders' equity	<u>42.049</u>	<u>26.774</u>

ORYZON GENOMICS SA
STATEMENTS OF OPERATIONS (UNAUDITED)
(US \$, amounts in thousands except per share data)

	<u>Three Months Ended September 30,</u>		<u>Nine Months Ended September 30</u>	
	<u>2017</u>	<u>2016</u>	<u>2017</u>	<u>2016</u>
Collaboration Revenue	0	252	20	785
Operating expenses:				
Research and Development	1.532	1.493	5.130	4.006
General and administrative	1.030	1.367	3.263	4.204
Total operating expenses	<u>2.561</u>	<u>2.860</u>	<u>8.393</u>	<u>8.210</u>
Loss from Operations	<u>-2.561</u>	<u>-2.608</u>	<u>-8.373</u>	<u>-7.425</u>
Other income, net	1.353	1.368	4.618	3.608
Net Loss	<u>-1.208</u>	<u>-1.239</u>	<u>-3.755</u>	<u>-3.818</u>
Net Financial & Tax	-169	-276	-844	-820
Net Result	<u>-1.376</u>	<u>-1.516</u>	<u>-4.599</u>	<u>-4.637</u>
Loss per share allocable to common stockholders:				
Basic	-0,04	-0,05	-0,11	-0,17
Diluted	-0,04	-0,05	-0,11	-0,17
Weighted average Shares outstanding				
Basic	33.491	27.589	31.176	27.517
Diluted	33.491	27.589	31.176	27.517

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.

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