

ORYZON Announces Encouraging Top-line Results from Phase I-IIA Clinical Trial in Acute Leukemia of RG6016 (ORY-1001) at the American Society of Hematology 2016 Annual Meeting

BARCELONA, SPAIN and CAMBRIDGE, MA, December 6th, 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 yesterday encouraging top-line results from the First in Man international multicenter Phase I clinical trial with RG6016 (a potent and selective inhibitor of lysine-specific histone demethylase-1, previously known as ORY-1001) in relapsed or refractory acute leukemia (RR-AL) patients (EUDRACT nº 2013-002447-29). Results were presented in a poster session at the 58th Annual Meeting of the American Society of Hematology (ASH) in San Diego, California.

The study met its primary and secondary endpoints, providing key information about the drug's safety, pharmacokinetics (PK) and pharmacodynamics (PD), supporting further clinical studies with RG6016 in patients with cancer.

RG6016 has been administered to 41 patients in total up to a maximum of three cycles. The drug showed oral bioavailability in patients with RR-AL, predictable pharmacokinetic parameters and was found to be well tolerated.

The study comprised an ascending dose (AD) phase, with 27 subjects recruited through 8 ascending cohorts, followed by an extension cohort (Phase IIA part of the study), where 14 patients with specific AML subtypes predicted to be more sensitive based upon preclinical studies were enrolled (AML MLL-translocated n=10, acute erythroleukemia/M6 n=4).

Data supportive of anti-leukemia activity include observations of morphologic differentiation, changes of gene expression patterns consistent with blast differentiation, and effects on blast counts in blood and bone marrow (BM). Importantly, in MLL gene fusion patients (n=6) there was evidence of morphologic blast differentiation in blood and/or BM in 67% of patients (4/6); from these, one patient showed blast BM reduction achieving a partial bone marrow response after 3 cycles and another patient showed blast cells cleared from blood & stable disease in BM. Two out of the 4 M6 patients enrolled exhibited a partial bone marrow response. On the overall, the four M6 patients were catalogued as stable bone marrow disease. Pharmacodynamic PD biomarkers have been identified that will permit monitoring of response to RG6016 treatment in future trials. All MLL gene fusion patients with evaluable PD samples showed evidence of blast differentiation assessed by qRT-PCR analysis.

“We are extremely pleased with these findings from our Phase I-IIA study of RG6016” commented Carlos Buesa, President and CEO of Oryzon Genomics. “Our drug is safe and well tolerated and exhibited a satisfactory PK profile. Furthermore, these results show that our selective LSD1 inhibitor at the

recommended dose promotes blast cell differentiation in individual patients. This, together with the partial BM responses observed, suggests that LSD1 inhibition may be a possible therapy for some AML patients and are supportive for further clinical trials”.

These top-line results from the study with RG6016 were presented by the company yesterday at a poster session at the 58th Annual Meeting of the American Society of Hematology (ASH) in San Diego, California, as well as during an investor and analyst luncheon hosted by the company on the same day. This event was webcasted, and an archived version of the webcast will be available on the company’s website at www.oryzon.com.

The data presented at ASH should be regarded as preliminary, as they remain subject to completion of the Clinical Study Report (CSR), expected by early 2017. Final results of the study will be presented in the appropriate scientific and medical forums following completion of the CSR.

About RG6016 (ORY-1001)

RG6016, previously known as ORY-1001, is a highly potent and selective oral epigenetic inhibitor that modulates LSD1, a histone demethylase that removes (“erases”) signals in the histone, provoking changes in the reading context of the chromosome and turning off genes. Aberrant “erasing” activity may lead to disease. LSD1 has been related with several malignancies such as solid tumours and haematological diseases. In Leukemia RG6016 affects AML stem cells, a sub-population of cancer cells that has been proposed to be responsible for frequent relapses of the disease. RG6016 also significantly reduces tumour cell load and increases survival time in mouse models of Acute Lymphoblastic Leukaemia. LSD1 inhibition has been proposed as a meaningful therapeutic option, alone or in combination, in several solid tumors such as SCLC. RG6016 is part of the worldwide collaboration signed between Oryzon and Roche on April 2014.

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 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.

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