

## ORYZON announces FDA Orphan Drug Designation granted to iadademstat for treatment of Acute Myeloid Leukemia

- **The compound is currently in Phase II**
- **Now has orphan designation in both U.S. and EU**

**MADRID, SPAIN and CAMBRIDGE, MA, UNITED STATES**, February 11th, 2021 – Oryzon Genomics, S.A. (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 the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to the company's first-in-class LSD1 inhibitor iadademstat for the treatment of patients with acute myeloid leukemia (AML). Iadademstat is an investigational, oral, small molecule covalent inhibitor of the epigenetic enzyme LSD1, a chromatin remodeler that interacts with a variety of transcription factors involved in leukemia and other cancers.

"Receiving Orphan Drug Designation for iadademstat in AML is an important recognition of the role that new drugs with new mechanisms of action may bring to this patient community, where we do not yet have any potentially curative medicines besides stem cell transplant. Iadademstat is showing a high overall response rate of 85% in our ongoing clinical Phase II study ALICE, with a rapid onset of action and with durable responses. In addition, we have seen a good safety and tolerability profile in the combination treatment with azacitidine. Future Phase II clinical trials in further combination treatments of AML are planned for the second half of this year," said Oryzon's Global Head of R&D and CSO, Dr. Torsten Hoffmann.

The FDA's Office of Orphan Drug Products grants orphan status to support the development of medicines for rare disorders that affect fewer than 200,000 people in the U.S. Orphan Drug Designation provides certain benefits, including market exclusivity upon regulatory approval if received, exemption of FDA application fees and tax credits for qualified clinical trials.

Iadademstat was previously granted orphan drug designation by the European Medicines Agency for the treatment of AML.

### **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. Oryzon has one of the strongest portfolios in the field. Oryzon's LSD1 program has rendered two compounds, vafidemstat and iadademstat, in Phase II clinical trials. In addition, Oryzon has ongoing programs for developing inhibitors against other epigenetic targets. Oryzon has a strong technological platform for biomarker identification and performs biomarker and target validation for a variety of malignant and neurological diseases. Oryzon has offices in Spain and the United States. Oryzon is one of the most liquid biotech stocks in Europe with +90 M shares negotiated in 2020 (ORY:SM / ORY.MC / ORYZF US OTC mkt). The company had a +25% stock performance in 2020 and its cash runway is expected to extend till 1Q2023. For more information, visit [www.oryzon.com](http://www.oryzon.com)

**About Iadademstat**

Iadademstat (ORY-1001) is a small oral molecule, which acts as a highly selective inhibitor of the epigenetic enzyme LSD1 and has a powerful differentiating effect in hematologic cancers (See Maes et al., Cancer Cell 2018 Mar 12; 33 (3): 495-511.e12.doi: 10.1016 / j.ccell.2018.02.002.). A first Phase I/IIa clinical trial with Iadademstat in refractory and relapsed acute leukemia patients demonstrated the safety and good tolerability of the drug and preliminary signs of antileukemic activity, including a CRi. Beyond hematological cancers, the inhibition of LSD1 has been proposed as a valid therapeutic approach in some solid tumors such as small cell lung cancer (SCLC), neuroendocrine tumors, medulloblastoma and others. Iadademstat has been tested in four clinical trials (two in monotherapy in SCLC and AML, and two in combination, in SCLC and AML) in more than 100 patients. In the combination studies, ALICE (ongoing), a Phase IIa trial in combination with azacitidine in elderly or unfit AML patients, and CLEPSIDRA (finalized), a Phase IIa trial in combination with platinum/etoposide in second line ED-SCLC patients, preliminary efficacy results have been reported.

**FORWARD-LOOKING STATEMENTS**

This communication contains, or may contain, forward-looking information and statements about Oryzon, 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 believes that the expectations reflected in such forward-looking statements are reasonable, investors and holders of Oryzon 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 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 to the Spanish Comisión Nacional del Mercado de Valores (CNMV), which are accessible to the public. Forward-looking statements are not guarantees of future performance and have not been reviewed by the auditors of Oryzon. 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 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 on the date hereof. Except as required by applicable law, Oryzon 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 or any other jurisdiction. Oryzon’s securities may not be offered or sold in the United States absent registration or an exemption from registration. Any public offering of Oryzon’s securities to be made in the United States will be made by means of a prospectus that may be obtained from Oryzon or the selling security holder, as applicable, that will contain detailed information about Oryzon and management, as well as financial statements.

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