

## ORYZON announces FDA Orphan Drug Designation granted to iadademstat for treatment of Small Cell Lung Cancer

- **Now has orphan drug designation for SCLC and AML**

**MADRID, SPAIN and CAMBRIDGE, MA, UNITED STATES, June 8th, 2022** - Oryzon Genomics, S.A. (ISIN Code: ES0167733015, ORY), a clinical-stage biopharmaceutical company leveraging epigenetics to develop therapies in diseases with strong unmet medical need, announced today that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation to the company's clinical stage LSD1 inhibitor iadademstat for the treatment of patients with small cell lung cancer (SCLC). Iadademstat is an oral, highly potent and selective inhibitor of the epigenetic enzyme LSD1, a chromatin remodeler that interacts with a variety of transcription factors involved in SCLC, other solid tumors and hematological cancers such as acute myeloid leukemia (AML).

"Receiving Orphan Drug Designation for iadademstat in SCLC is an important recognition of the role that targeted drugs with new mechanisms of action may bring to this patient community, where we do not yet have any potentially curative medicines", said Dr. Douglas Faller, Oryzon's Global CMO. "Iadademstat is a novel epigenetic targeted approach to the treatment of this rapidly fatal tumor. The drug has two independent and complementary actions against SCLC – iadademstat epigenetically reprograms the genome of the tumor cell, while also greatly increasing the ability of the patient's own immune system to recognize and destroy the cancer. Prior clinical studies of iadademstat have demonstrated its activity and manageable safety profile in the treatment of SCLC."

Dr. Carlos Buesa, Oryzon's CEO, said: "We are really excited with the new Orphan Drug designation for iadademstat in SCLC by the FDA. This will facilitate and expedite our development of this targeted agent in this disease with great unmet medical need."

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 FDA and 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 leader in epigenetics. Oryzon has one of the strongest portfolios in the field, with two LSD1 inhibitors, iadademstat and vafidemstat, in Phase II clinical trials, and other pipeline assets directed against other epigenetic targets. In addition, Oryzon has a strong platform for biomarker identification and target validation for a variety of malignant and neurological diseases. 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 FiM Phase I/IIa clinical trial with Iadademstat in R/R AML patients demonstrated the safety and good tolerability of the drug and preliminary signs of antileukemic activity, including a CRi (see Salamero et al, *J Clin Oncol*, 2020, 38(36): 4260-4273. doi: 10.1200/JCO.19.03250). In an ongoing Phase IIa trial in elder 1L-AML patients (ALICE trial), Iadademstat has shown encouraging safety and efficacy data in combination with azacitidine (see Salamero et al., ASH 2021 poster). The company has recently obtained approval from the U.S. FDA for its IND for FRIDA, a Phase Ib trial of Iadademstat plus gilteritinib in patients with relapsed/refractory AML with FLT3 mutations. 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 (NET), medulloblastoma and others. In a Phase IIa trial in combination with platinum/etoposide in second line ED-SCLC patients (CLEPSIDRA trial), preliminary activity and safety results have been reported (see Navarro et al., ESMO 2018 poster). New trials in combination in SCLC and NET are under preparation. In total Iadademstat has been dosed so far to more than 100 cancer patients in four clinical trials.

### **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.

#### **IR, US**

Ashley R. Robinson  
LifeSci Advisors, LLC  
+1 617 430 7577  
arr@lifesciadvisors.com

#### **IR & Media, Europe**

Sandya von der Weid  
LifeSci Advisors, LLC  
+41 78 680 05 38  
svonderweid@lifesciadvisors.com

#### **Spain**

Patricia Cobo  
/ Carlos C. Ungría  
+34 91 564 07 25  
pcobo@atrevia.com  
cungria@atrevia.com

#### **Oryzon**

Saikat Nandi  
Chief Business Officer  
+1 917 208 8293  
snandi@oryzon.com