

World Rare Disease Day**ORYZON committed to rare diseases with its research in acute myeloid leukemia**

- ❖ **Ongoing Phase II ALICE clinical trial with iadademstat shows efficacy in elderly patients with acute myeloid leukemia**
- ❖ **Obtained in 2013 the designation of iadademstat as an orphan drug for AML by the European Medicines Agency**
- ❖ **Also exploring genetic variants of autism and schizophrenia**

**MADRID, SPAIN and CAMBRIDGE, MA, UNITED STATES, February 28<sup>th</sup>, 2020** - 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, continues to contribute to the fight against rare diseases, whose world day is celebrated on February 29th, through its research on acute myeloid leukemia (AML).

Oryzon Genomics has since long been involved in the research on AML, a disease with low prevalence and within the so-called rare diseases. The company is performing a Phase II clinical trial (ALICE study) in AML with its drug iadademstat, a selective LSD1 inhibitor, for which it obtained the designation of orphan drug for the treatment of AML by the European Medicines Agency in 2013. Last December, Oryzon presented at the 61st Annual Conference of the American Society of Hematology, ASH-2019, in Orlando, Florida, USA, new data from ALICE, which showed clinical efficacy in AML patients.

In these results presented at ASH-2019, a high number of positive objective responses (OR) were reported, in 6 of 8 evaluable patients (75% OR): of these, there were 2 complete remissions (CR), 3 complete remissions with incomplete hematologic recovery (CRi) and 1 partial remission (PR). The average follow-up time among evaluable patients was 20 weeks, with an average time to response (TTR) of only 32 days in those patients who responded. Two of the 5 patients (40%) who had received more than 3 treatment cycles had also become transfusion independent (that is, they did not require periodic red blood cell transfusions). The company plans to provide a new update of the results of this study at the European Hematology Association conference (EHA-2020) to be held in Frankfurt in June.

ALICE is a single-arm Phase II study that evaluates the safety and clinical efficacy of iadademstat in combination with standard of care azacitidine in newly diagnosed elderly AML patients, who are not eligible for conventional therapy. The study is being carried out in several Spanish hospitals.

Oryzon's commitment to rare diseases is not limited only to the cancer field but also expands to neurological diseases. Thus, the company is beginning the evaluation of the potential of its drug

vafidemstat, a CNS-optimized LSD1 inhibitor, as a therapeutic alternative in rare genetic variants of autism and schizophrenia. These possibilities have recently been raised by several independent studies of prestigious American Universities that have shown the efficacy of Oryzon LSD1 inhibitors in various experimental models of these genetic subvariants, which could represent a precision medicine approach in the psychiatric field that would be the first of its kind.

### **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 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 neurodegenerative diseases. Oryzon has offices in Spain and the United States. For more information, visit [www.oryzon.com](http://www.oryzon.com)

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