ORYZON presents new robust Phase II iadademstat efficacy data in AML at ASH-2020

- **Robust signals of clinical efficacy, with ORR of 85%, of which 64% are CR/CRi**
- **Longest remission to date 690 days, still ongoing**
- **86% of CR/CRi show a duration of response of more than 6 months**
- **Iadademstat and azacitidine combination shows a good safety profile**

MADRID, SPAIN and CAMBRIDGE, MA, UNITED STATES, December 7th, 2020 –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, presents new positive efficacy data from its ongoing Phase II ALICE trial, which is investigating iadademstat in combination with azacitidine in elderly patients with acute myeloid leukemia (AML). The data were presented at the virtual 62th Congress of the American Society of Hematology, ASH-2020, in an e-poster entitled "Robust Efficacy Signals in elderly AML Patients treated with Iadademstat in Combination with Azacitidine (ALICE Phase IIa Trial)".

The evidence of clinical efficacy continues to be robust and consistent with previously reported data, with an objective response rate (ORR) of 85% (11 out of 13 evaluable patients); of these, 64% were complete remissions (7 CR/CRi), and 36% partial remissions (4 PR). Mean Time to Response (TTR) was only 34 days. With historical response rates of 27% in this population when treated with azacitidine alone, these results support strong synergies between iadademstat and azacitidine when used in combination.

The duration of the observed responses is encouraging, with 86% of the CR/CRi lasting more than 6 months, with a current median duration of 308 days. The current longest remission is 690 days and still continuing. Another patient who was treated for only 20 weeks achieved CR and was transfusion independent for a total of 77 weeks. A third patient, who had achieved CR and was also transfusion independent, died due to Covid-19 infection at week 48. Those patients with longer treatment periods have also improved or overcome their dependency on blood transfusions. The results obtained so far suggest that the therapeutic efficacy between the two iadademstat doses used in the study is equivalent, with current ORRs of 85% at 90 ug/sqm/d and 83% at 60 ug/sqm/d.

Dr. Carlos Buesa, Oryzon's CEO, said: "These latest results from the Phase II ALICE trial are very impressive. A robust percentage of responses continue to compare well with approved therapies and, very importantly, there is a clear trend to mature longer responses. Considering the different mechanisms of action of proapoptotic BCL2 inhibitors and the pro-differentiating agent iadademstat, we believe that combination approaches with iadademstat might increase therapeutic options for patients in first line, as well as for refractory or intolerant patients who have received BCL2 inhibitors as first line."

The combination of iadademstat with azacitidine continues to show a benign safety profile. Besides the expected hematological impact, in line with the pharmacologic mode of action and previously presented at EHA-2020, the combination continues to appear safe and well tolerated by elderly AML patients.

Three additional patients have been included in the study since submission of the e-poster (November 17th). Due to the cut-off, their cases have not been included in these latest data.

The objective of the ALICE trial is to inform the broader use of iadademstat in leukemia. ALICE is designed as a single-arm, open-label study of iadademstat in combination with the standard of care treatment azacitidine in newly diagnosed elderly AML patients and is being carried out in five Spanish hospitals. The study is divided into two parts, the first optimizing the dose of the combination, and the second evaluating the combination's effectiveness. Efficacy endpoints include clinical response, as well as time to response, duration of response and average survival. At the time of writing the ASH-2020 poster, 18 patients had been enrolled in this trial; the study will recruit up to a maximum of 36 patients. Following interruptions in recruitment due to the Covid-19 pandemic, recruitment has now resumed at the expected rate.

A copy of the poster is available here

For more information about ASH-2020, please visit ASH's website

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 neurological diseases. Oryzon has offices in Spain and the United States. For more information, visit 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), medulloblastoma and others. Oryzon is conducting two Phase IIa clinical trials of iadademstat in combination; the first one in combination with azacitidine in elderly AML patients (ALICE study) and the second one in combination with platinum/etoposide in second line SCLC patients (CLEPSIDRA study). In both studies, preliminary clinical results have been reported.

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