# ORYZON starts preclinical collaboration on Kabuki syndrome with Kennedy Krieger Institute and Johns Hopkins University

MADRID, SPAIN and CAMBRIDGE, MA, UNITED STATES, May 3rd, 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 the start of a preclinical collaboration on Kabuki Syndrome with researchers from Kennedy Krieger Institute and Johns Hopkins University led by Dr. Jacqueline Harris, Director of the Epigenetics Clinic at Kennedy Krieger Institute and an assistant professor in pediatrics, neurology and genetics at the Johns Hopkins University School of Medicine and Dr. Hans Bjornsson, Founder of the Epigenetic and Chromatin Clinic and associate professor of pediatrics and genetics at the Johns Hopkins University School of Medicine.

Kabuki syndrome (KS) is an autosomal dominant/X-linked disorder that affects multiple organ systems including neuro, immune, auditory and cardiac systems. Patients show characteristic distinctive facial features, growth retardation, and mild to moderate intellectual disability and autoimmune disorders. The majority (>70%) of molecularly confirmed cases of KS have loss-of-function variants in KMT2D gene. This gene, aka MLL2, catalyzes the addition of methyl groups to lysine 4 of histone 3, which are marks associated with open chromatin, thus regulating the expression of critical target genes.

### **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 <a href="https://www.oryzon.com">www.oryzon.com</a>

#### **About Vafidemstat**

Vafidemstat (ORY-2001) is an oral, CNS optimized LSD1 inhibitor. The molecule acts on several levels: it reduces cognitive impairment, including memory loss and neuroinflammation, and at the same time has neuroprotective effects. In animal studies vafidemstat not only restores memory but reduces the exacerbated aggressiveness of SAMP8 mice, a model for accelerated aging and Alzheimer's disease (AD), to normal levels and also reduces social avoidance and enhances sociability in murine models. In addition, vafidemstat exhibits fast, strong and durable efficacy in several preclinical models of multiple sclerosis (MS). Oryzon has performed two Phase IIa clinical trials in aggressiveness in patients with different psychiatric disorders (REIMAGINE) and in aggressive/agitated patients with moderate or severe AD (REIMAGINE-AD), with positive clinical results reported in both. Additional finalized Phase IIa clinical trials with vafidemstat include the ETHERAL trial in patients with Mild to Moderate AD, where a significant reduction of the inflammatory biomarker YKL40 has been observed after 6 and 12 months of treatment, and the pilot, small scale SATEEN trial in Relapse-Remitting and Secondary Progressive MS, where antiinflammatory activity has also been observed. Vafidemstat has also been tested in a Phase II in severe Covid-19 patients (ESCAPE) assessing the capability of the drug to prevent ARDS, one of the most severe complications of the viral infection, where it showed significant anti-inflammatory effects in severe Covid-19 patients. Currently, vafidemstat is in two Phase IIb trials in borderline personality disorder (PORTICO) and in schizophrenia patients (EVOLUTION). The company is also deploying a CNS precision medicine approach with vafidemstat in genetically-defined patient subpopulations of certain CNS disorders and is preparing a clinical trial in Kabuki Syndrome patients that is expected to start in 1H 2022. The company is also exploring the clinical development of vafidemstat in other neurodevelopmental syndromes.

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