ORYZON announces clinical candidate nomination of ORY-4001 for the treatment of CNS disorders

- A highly selective HDAC-6 inhibitor
- Rescues motor-neuronal deficiencies in a Charcot-Marie-Tooth model
- To start IND enabling studies

MADRID, SPAIN and BOSTON, MA, UNITED STATES, March 13th, 2023 - 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 nomination of ORY-4001, a selective histone deacetylase 6 (HDAC-6) inhibitor, as clinical development candidate for the treatment of certain neurological diseases as Charcot-Marie-Tooth (CMT), Amyotrophic Lateral Sclerosis (ALS) and others. HDAC6 inhibitors have been previously proposed as potentially effective treatments for CMT, ALS and other neurological disorders that lack effective treatments.

In 2022, Oryzon and the CMT Research Foundation (CMTRF), a U.S.-based patient-led, non-profit organization focused on delivering treatments and cures for CMT, entered into an agreement to explore Oryzon's HDAC-6 inhibitors. As a result of positive preclinical results obtained under this collaboration, the company has selected a candidate for clinical development, ORY-4001. This compound is a HDAC6 inhibitor with excellent pharmacology and very high selectivity against other HDAC-classes, resulting in a remarkable safety profile avoiding hematoxicity. The compound exhibits strong anti-inflammatory properties and positive in-vivo data in inflammatory models. ORY-4001 has shown multiple positive responses in a validated CMT1A peripheral neuropathy in vivo model which reliably recapitulates many of the symptoms of this condition in humans. ORY-4001 will enter now into IND enabling studies to prepare the compound for clinical studies.

Dr. Jordi Xaus, Oryzon's CSO, said: "The nomination of ORY-4001 as a new candidate for clinical development is a clear confirmation of the excellence of the Epigenetic Discovery Programs at Oryzon complementing and expanding our existing LSD1 programs. ORY-4001 high selectivity and remarkable safety inhibiting HDAC6 anticipates a broad applicability in peripheral neuropathies as CMT or ALS where there is an enormous medical need."

CMT is a progressive, degenerative disease involving the peripheral nerves. It affects 150,000 Americans and more than 3 million people around the world. It is one of the most prevalent conditions among rare diseases and currently lacks effective treatments or cures. CMT is caused by a variety of genetic mutations. CMT1A is the most prevalent form of all, accounting for approximately half of all people with CMT.

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About Oryzon

Founded in 2000 in Barcelona, Spain, Oryzon (ISIN Code: ES0167733015) is a clinical stage biopharmaceutical company and the European leader in epigenetics, with a strong focus on personalized medicine in CNS disorders and oncology. Oryzon's team is composed of highly qualified professionals from the pharma industry located in Barcelona, Boston, NYC and San Diego. Oryzon has an advanced clinical portfolio with two LSD1 inhibitors, vafidemstat in CNS, and iadademstat in oncology in several Phase II clinical trials. The company has 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

FORWARD-LOOKING STATEMENTS

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