

Positive preclinical data of ORY-4001 for the treatment of Charcot-Marie-Tooth (CMT) disease presented at the 2023 Peripheral Nerve Society (PNS) Annual Meeting

- ❖ **Comprehensively reversed disease progression symptoms in a CMT mice model**
- ❖ **Selected for an oral presentation**
- ❖ **A highly selective and safe HDAC-6 inhibitor**
- ❖ **To start IND-enabling studies soon**

MADRID, SPAIN and BOSTON, MA, UNITED STATES, June 21th, 2023 - Oryzon Genomics, S.A. (ISIN Code: ES0167733015, ORY), a clinical-stage biopharmaceutical company leveraging epigenetics to develop therapies in diseases with a strong unmet medical need, presented yesterday positive preclinical efficacy data of ORY-4001, a selective histone deacetylase 6 (HDAC-6) inhibitor, in an oral communication at the 2023 Peripheral Nerve Society annual meeting (PNS-2023) held in Copenhagen from 17-20 June 2023. ORY-4001 treatment was able to reverse disease progression symptoms in a Charcot-Marie-Tooth (CMT) mice model.

The presented results are the fruit of a collaboration agreement between Oryzon and the CMT Research Foundation (CMTRF), a U.S.-based patient-led, non-profit organization focused on delivering treatments and cures for CMT, which was entered in 2022 to explore the therapeutic potential of Oryzon's HDAC-6 inhibitors. ORY-4001 was able to reverse in a dose-dependent manner the CMT1A phenotypes. Notably, ORY-4001 was able to improve myelination and restore axon integrity in the sciatic nerve, and improved compound muscle action potential and nerve conduction in comparison with untreated animals. Remarkably, ORY-4001-treated animals displayed a significant body weight recovery, suggesting an overall physiological improvement. The doses used in this study were safe and below the maximal tolerated dose, which allows room for dose increases in additional studies to further deepen the therapeutic benefit.

ORY-4001 is an HDAC6 inhibitor with excellent pharmacology and very high selectivity against other HDAC classes, resulting in a remarkable safety profile avoiding hematotoxicity. The compound exhibits also strong anti-inflammatory properties and positive in-vivo data in inflammatory models. ORY-4001 will enter now into IND enabling studies to prepare the compound for clinical studies.

ORY-4001 was recently nominated as a clinical development candidate for the treatment of certain neurological diseases such as 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.

Dr. Jordi Xaus, Oryzon's CSO, said: "We are honored to have been selected for an oral presentation. This highlights the interest of the community in the study results, which strongly suggest a clear therapeutic potential in peripheral neuropathies such as CMT, where there is an enormous medical need. We are exploring with the CMT community how we can expedite this program to the clinic."

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, accounting for approximately half of all people with CMT.

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

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