

ORYZON to present positive preclinical efficacy data of ORY-4001 in Charcot-Marie-Tooth (CMT) disease at the 2023 Peripheral Nerve Society (PNS) Annual Meeting

- ❖ Selected for Oral Presentation
- ❖ Study done in the CMT1A mouse model
- ❖ Supported by the CMT Research Foundation

MADRID, SPAIN and BOSTON, MA, UNITED STATES, May 30th, 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, announced today that it will present positive preclinical efficacy data of ORY-4001 in a CMT model at the 2023 Peripheral Nerve Society (PNS) Annual Meeting, which will be held June 17-20 at the Bella Center in Copenhagen, Denmark. The scientific committee of the 2023 PNS Annual Meeting has selected “*ORY-4001, a Novel Potent and Selective Oxadiazole-Based HDAC6 Inhibitor Shows Pre-Clinical Therapeutic Efficacy in CMT1A*” for an oral presentation, which will take place on Tuesday, June 20, at 08:40 am CEST.

In 2022, Oryzon and the (CMTRF), a U.S.-based patient-led, non-profit organization focused on delivering treatments and cures for CMT, entered into an agreement to explore the therapeutic potential of Oryzon’s histone deacetylase 6 (HDAC-6) inhibitors. Following positive preclinical results obtained under this collaboration, Oryzon 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 hematotoxicity. The compound exhibits strong anti-inflammatory properties and positive in-vivo data in inflammatory models. In the study to be presented at the 2023 PNS Annual Meeting, ORY-4001 showed multiple positive responses in a validated CMT1A peripheral neuropathy in vivo model which effectively mimics many of the symptoms of this condition in humans.

Dr. Jordi Xaus, Oryzon’s CSO, said: “Although this is only a first proof-of-concept preclinical model of a peripheral neuropathy, the robustness of the data obtained in this CMT1A model is really encouraging and prompts us to intensify our efforts to pursue IND enabling studies to support first-in- human administration of ORY-4001 in the following quarters.”

CMT is a progressive, degenerative disease that causes damage to 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.

For more information about PNS-2023, please visit the [PNS-2023 website](#).

[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, iadamemstat 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 www.oryzon.com

About ORY-4001

ORY-4001 is a highly selective histone deacetylase 6 (HDAC-6) inhibitor. Due to its positive pharmacological properties, it was recently nominated as a clinical development candidate for the treatment of certain neurological diseases such as Charcot-Marie-Tooth disease (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.

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This communication contains, or may contain, forward-looking information and statements about Oryzon, including financial projections and estimates and their underlying assumptions, statements regarding plans, objectives and expectations with respect to future operations, capital expenditures, synergies, products and services, and statements regarding future performance. Forward-looking statements are statements that are not historical facts and are generally identified by the words “expects,” “anticipates,” “believes,” “intends,” “estimates” and similar expressions. Although Oryzon believes that the expectations reflected in such forward-looking statements are reasonable, investors and holders of Oryzon shares are cautioned that forward-looking information and statements are subject to various risks and uncertainties, many of which are difficult to predict and generally beyond the control of Oryzon that could cause actual results and developments to differ materially from those expressed in, or implied or projected by, the forward-looking information and statements. These risks and uncertainties include those discussed or identified in the documents sent by Oryzon to the Spanish Comisión Nacional del Mercado de Valores (CNMV), which are accessible to the public. Forward-looking statements are not guarantees of future performance and have not been reviewed by the auditors of Oryzon. You are cautioned not to place undue reliance on the forward-looking statements, which speak only as of the date they were made. All subsequent oral or written forward-looking statements attributable to Oryzon or any of its members, directors, officers, employees or any persons acting on its behalf are expressly qualified in their entirety by the cautionary statement above. All forward-looking statements included herein are based on information available to Oryzon on the date hereof. Except as required by applicable law, Oryzon does not undertake any obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise. This press release is not an offer of securities for sale in the United States or any other jurisdiction. Oryzon’s securities may not be offered or sold in the United States absent registration or an exemption from registration. Any public offering of Oryzon’s securities to be made in the United States will be made by means of a prospectus that may be obtained from Oryzon or the selling security holder, as applicable, that will contain detailed information about Oryzon and management, as well as financial statements.

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