ORYZON achieves milestone in the clinical development of ORY-1001

Multiple ascending dose stage of its Phase I Clinical Trial in patients with relapsed or refractory acute leukemia concluded and milestone payment achieved

BARCELONA, SPAIN / CAMBRIDGE MA, 7 September 2015. Oryzon Genomics has finalized the multiple ascending dose (MAD) stage of its Phase I clinical trial to evaluate the safety, tolerability and pharmacokinetics of ORY-1001 in patients with relapsed or refractory acute leukemia (AL). ORY-1001 is a potent and selective inhibitor of Lysine Specific Demethylase 1 (LSD1). Through the establishment of a Recommended Dose (RD) of ORY-1001, Oryzon has achieved this development milestone included in the License Agreement with Roche signed in April 2014 and will receive a payment of USD 4 million.

The preliminary results obtained so far demonstrate the safety and tolerability of ORY-1001 in patients suffering from AL. An extension arm will now commence to evaluate the preliminary efficacy of ORY-1001 in various subpopulations of acute myeloid leukemia (AML), including mixed lineage leukemia (MLL).

"*We have done intense work during the last year to understand how ORY-1001 is tolerated and behaves in leukemia patients; now we enter the next stage with the extension arm in genetically selected patients to determine clinical responses with the hope that this novel drug and mechanism of action can make a difference in these patients*" said Dr. Cesar Molinero Chief Medical Officer of ORYZON.

AML is a blood cancer arising from the myeloid lineage of haematopoietic stem cells. Most patients with AML die from progressive disease after relapse, which is associated with a small sub-fraction of leukemic cells termed leukemic stem cells (LSC). Drugs which selectively target the LSC compartment with minimum toxicity to the normal HSC compartment are a novel approach. In preclinical studies, LSD1 has been shown to sustain the oncogenic potential of MLL-AF9 LSC and therefore inhibiton of LSD1 is a good target to test this therapeutic approach.

ABOUT ORY-1001

ORY-1001 is a highly selective and potent LSD1 inhibitor which is orally administered to patients. ORY-1001 affects AML stem cells, a sub-population of cancer cells that has been proposed to be responsible for frequent relapses of the disease. ORY-1001 significantly reduces tumor cell load and increases survival time in mouse models of Acute Lymphoblastic Leukaemia. LSD1 is also associated with other malignancies such as solid tumors and other haematological diseases.

ABOUT ORYZON

Founded in 2000 in Barcelona, Spain, Oryzon (www.oryzon.com) is a privately held, clinical stage biopharmaceutical company considered as the European champion in Epigenetics with one of the strongest

portfolios in the field. Its LSD1 program is currently covered by 19 patent families and has rendered one compound in clinical trials and another one is anticipated to enter clinical trials in early 2016. In addition, Oryzon has ongoing programs for developing inhibitors against other histone demethylases and histone methyltransferases. The company has also a strong technological platform for biomarker identification and performs biomarker and target validation for a variety of malignant and neurodegenerative diseases. Oryzon's strategy is to develop first in class compounds against novel epigenetic targets through Phase II clinical trials, at which point it is decided on a case-by-case basis to either keep the development in-house or to partner or out-license the compound for late stage development and commercialization. The opening of Oryzon's U.S. operations in Cambridge, Massachusetts, was announced in October 2014.

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