Oryzon obtains orphan designation for ORY-1001 for the treatment of Acute Myeloid Leukemia from the European Medicines Agency

Barcelona, July 15th, 2013. Oryzon announced today that it has received from EMA the orphan designation for ORY-1001, an inhibitor of LSD1 for the treatment of Acute Myeloid Leukemia.

Barcelona, July 15th, 2013. Oryzon has received from the Committee for Orphan Medicinal Products (COMP), the committee at the European Medicines Agency that is responsible for reviewing applications for 'orphan-medicinal-product designation', the positive opinion on its application for orphan designation of ORY-1001 for the treatment of Acute Myeloid Leukemia (AML) after the meeting held by the COMP in London on July 9-11.

ORY-1001 is an extremely selective inhibitor of Lysine Specific Demethylase-1 (LSD1, aka KDM1), an epigenetic modulator that regulates gene expression by demethylating specifically some lysines in the histones. LSD1 forms part of protein complexes involved in transcriptional regulation, and misregulation of these transcriptional complexes may result in disease.

ORY-1001 is an orally-active subnanomolar specific LSD1 inhibitor that reduces leukemic stem cell potential, potently inhibits colony formation, overcomes the differentiation block in AML cell lines, and induces apoptosis / inhibits proliferation at sub-nanomolar concentrations in selected AML cell lines.

ORY-1001 has successfully passed the regulatory toxicology studies and is now entering into a Phase I clinical study on acute leukemia patients. Besides hematological cancers, many academic groups have published reports pointing for a key role of this mechanism of action in some specific solid tumors.

The orphan designation is for medicines to be developed for the treatment of rare diseases that are life-threatening or very serious and for which no satisfactory method of treatment of the condition concerned is authorised, or, if such a method exists, the medicine will be of significant benefit to those affected by the condition. In the European Union (EU), a disease is defined as rare if it affects fewer than 5 in 10,000 people across the EU.

This designation for ORY-1001 recognizes the scientific rationale of this approach for the possible treatment of this deadly disease but also will entitle ORYZON, as sponsor, some advantages like:

- Market exclusivity: For 10 years after the granting of a marketing authorisation (approval for sale), orphan medicinal products benefit from market exclusivity in the EU. During that period, directly competitive similar products cannot normally be placed on the market.

- Protocol assistance The Agency can provide scientific advice to optimise development and guidance on preparing a dossier that will meet European regulatory requirements. This helps small companies like ORYZON to maximise the chances of their marketing authorisation application being successful.
- Fee reductions. A special fund from the European Commission, agreed annually by the European Parliament, is used by the Agency to grant fee reductions. Reduction of fees will be considered for various centralised activities, including applications for marketing authorisation, inspections and protocol assistance. Additional fee reductions apply for small and medium-sized enterprises (SMEs) like ORYZON .
- EU-funded research Sponsors developing orphan medicinal products may be eligible for grants from EU and Member State programmes and initiatives supporting research and development, including the Commission's framework programme.

Acute Myeloid Leukaemia (AML) is a blood cancer arising from the myeloid lineage of haematopoietic stem cells. Most patients with acute myeloid leukaemia (AML) die from progressive disease after relapse, which is associated with a small sub-fraction of leukemic cells termed leukemic stem cells (LSC). Current therapeutics target only the rapidly proliferating leukemic progenitors, and not the more chemoresistant LSC. Therefore the pharmaceutical industry is making a serious effort to develop drugs which selectively target the LSC compartment with minimum toxicity to the normal HSC compartment. Histone demethylase LSD1/KDM1A has been proven to sustain the oncogenic potential of MLL-AF9 leukemia stem cells and it is therefore a good target to test this therapeutic approach.

Epigenetics is a hot spot field in the pharmaceutical industry. It is predicted that world revenues for epigenetic therapies and technologies will reach $2.73bn in 2015 and that the overall market will grow with a CAGR of 16% between 2010 and 2015. Therapies will remain the largest source of revenue in the epigenetics market. The deal activity on the field is intense.

Oryzon Genomics is the global leader in Histone Lysine Demethylases with an special emphasis on Lysine Specific Demethylases (LSD1 and LSD2). LSD1 has been proposed as a target for oncology, viral diseases and neurodegeneration. Oryzon has a wide drug-discovery program on LSD1 with around 900 compounds and two preclinical candidates. According to Carlos Buesa, C.E.O. of the company. "*This designation will help us on the clinical development of our compound as we have identified now a subset of diseases in which this mechanism looks particularly efficient. We are really excited to test clinically the potential of LSD1 as therapeutic target in hematological cancers. For any company willing to play a role in these indications we are the partner of choice*"

About Oryzon

Founded in 2000, Oryzon (www.oryzon.com) has one of the most complete technological platforms for biomarker identification in Europe. With a strong specialization in genomics, proteomics and bioinformatics, the company identifies biomarkers for a variety of neoplasic and neurodegenerative diseases. The company has a powerful platform for biomarker and target validation which includes technologies such as RNAi, microarrays, phage display and a structural genomic platform with a fragment screening approach (NMR and X ray crystallography).

For further information, please contact:

Emili Torrell
Business Development Officer
Tel. +34 93 515 13 13,
etorrell@oryzon.com