Onxeo announces positive DSMB recommendation to continue Livatag® ReLive study in HCC

Paris (France), Copenhagen (Denmark), April 4, 2016 – Onxeo S.A. (Euronext Paris, Nasdaq Copenhagen: ONXEO), an innovative company specializing in the development of orphan oncology therapeutics, today announced that it has received a unanimous recommendation from the Data Safety Monitoring Board (DSMB), the independent European board of experts that monitors the safety of the Livatag® Phase III trial, "ReLive", to continue the study without modification.

As per study protocol, the DSMB meets twice a year to review the safety data of the ReLive trial and subsequently issues recommendations on the conduct of the study.

The positive DSMB recommendation granted for the eighth time further confirms the acceptable safety profile of Livatag®.

ReLive is an ongoing international, randomized Phase III trial designed to evaluate the efficacy of intravenous (IV) administration of Livatag® in patients with advanced hepatocellular carcinoma (HCC, primary liver cancer) after failure or intolerance to sorafenib. The study plans to enroll a total of 400 patients across approximately 90 sites. To date, more than 65% of the patients have been randomized in the study.

The DSMB reviews the safety data of the treated patients in the study, totaling more than 600 infusions of Livatag®.

"This eigth positive recommendation from our independent board of experts once again confirms Livatag®'s safety profile, based on data collected from a growing number of patients. We remain very confident in the potential of this innovative product based on a novel nanoparticle formulation, allowing to overcome tumor resistance to traditional chemotherapy. It could represent a significant breakthrough in the treatment of hepatocellular carcinoma, a very severe cancer with a high unmet medical need," commented Judith Greciet, CEO of Onxeo.

About hepatocellular carcinoma

Hepatocellular carcinoma (HCC) or hepatocarcinoma is the most common of the primary liver cancers (85% to 90%). According to Globocan (2012 data), liver cancer is the sixth most common cancer in terms of incidence (782,000 new cases worldwide each year, 5.6% of all new cancer cases) with the second highest mortality rate (746,000 deaths, 9.1% of the total) after lung cancer. The risk factors are well known: infection by hepatitis viruses (B and C), overconsumption of alcohol (another major cause of cirrhosis) and metabolic diseases, especially obesity, a growing cause of cirrhosis and HCC.

About ReLive

ReLive is an international Phase III trial designed to assess Livatag®'s efficacy on survival in 400 patients with advanced hepatocellular carcinoma (HCC) following treatment after failure or intolerance to Sorafenib. The trial is ongoing in 11 countries (Europe, US, MENA region). The recruitment rate is in line with expected timelines of issuing preliminary outcomes of the Phase III study by mid 2017.

About Livatag®(doxorubicin Transdrug™)

Livatag® (Doxorubicin Transdrug™) is a doxorubicin formulation in the form of lyophilized nanoparticles of polyisohexylcyanoacrylate (PIHCA).This new therapeutic approach allows drug resistance to be avoided by short-circuiting the mechanisms of multi-drug resistance developed by tumor cells through the masking of the anticancer agent. Acting as a 'Trojan horse,' the nanoparticle formulation avoids rejection of doxorubicin outside the cell so that it can exert its cytotoxic action. By specifically targeting tumor cells in the liver and overcoming resistance to doxorubicin, Livatag® represents a significant breakthrough in the treatment of this cancer. The first indication of this product is hepatocellular carcinoma; the sixth most widespread cancer in the world and the second cause of cancer-related death.Livatag® is also being evaluated within a comprehensive preclinical evaluation program, to explore potential combinations with immuno-oncology agents (such as checkpoint inhibitors), cytotoxic agents and targeted therapies and expand product potential, with a primary focus on solid tumors

About Onxeo

Onxeo is a leading developer of orphan oncology drugs. The Company is focused on developing innovative therapeutics for rare cancers, one of the fastest growing markets in the healthcare industry with high, unmet medical needs. Onxeo's comprehensive portfolio features a broad orphan oncology pipeline, with three independent programs in advanced clinical development, including Onxeo's first approved orphan oncology drug, Beleodaq®. In addition, Onxeo has successfully developed and registered two non-cancer products which are currently being commercialized in the U.S. and Europe. Onxeo's vision is to become a global leader and pioneer in oncology, with a focus on orphan or rare cancers, by developing advanced, effective, and safe therapeutics designed to improve the lives of patients. The Company is headquartered in Paris, France and has approximately 50 employees. Onxeo is listed on Euronext in Paris, France (Ticker: ONXEO, ISIN Code: FR0010095596) and Nasdaq Copenhagen, Denmark (Ticker: ONXEO).

Onxeo orphan oncology products at the advanced development stage are:

- **Livatag®** (Doxorubicin Transdrug™): Currently being evaluated in a Phase III trial (ReLive) in patients with hepatocellular carcinoma (primary liver cancer); and in combination with other cancer agents in first-line HCC
- **Beleodaq®** (belinostat): FDA-approved in the U.S. in 2014 under the agency's accelerated approval program as a second-line treatment for patients with peripheral T-cell lymphoma (PTCL) and currently marketed by Onxeo's partner in the U.S., Spectrum Pharmaceuticals; belinostat in combination with other cancer agents is currently in development in first-line treatment for patients with PTCL (BelCHOP) and in other solid tumors
- **AsiDNA** : the first-in-class siDNA (signal interfering DNA) which has successfully undergone a proof-of-concept Phase 1/2a trial in metastatic melanoma
- **Validive®** (Clonidine Lauriad®): Positive final results from a Phase II trial in head and neck cancer patients with severe oral mucositis;

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