Onxeo Completes Enrollment in Phase III Study of Livatag® for the Treatment of Hepatocellular Carcinoma

Milestone completion in line with expected preliminary outcomes mid-2017

Paris (France), Copenhagen (Denmark), January 24, 2017 – 18:30 CET – Onxeo S.A. (Euronext Paris, NASDAQ Copenhagen: ONXEO), a biopharmaceutical company specializing in the development of innovative drugs for the treatment of orphan diseases, in particular in oncology, today announced the completion of enrollment in ReLive, the international Phase III clinical trial evaluating Livatag® for the treatment of advanced hepatocellular carcinoma (HCC).

"Advanced HCC is a particularly severe form of liver cancer with a high mortality and very few treatment options", commented Pr. Philippe Merle, Professor in Hepatology (La Croix Rousse Hospital, Lyon, France) and Principal Investigator of the ReLive study. *"All patients have now been enrolled in the Phase III trial of Livatag® and the DSMB recommendations point towards an adequte safety profile. We hope this treatment will become a new option for patients with advanced HCC for whom there is a strong unmet therapeutic need and we are now looking forward to the preliminary efficacy data expected within a few months."*

The ReLive trial is evaluating the efficacy of intravenous (IV) administration of Livatag® (doxorubicin transdrug®) in patients with advanced HCC after failure or intolerance to sorafenib compared to the best standard of care chosen by the physician. In line with Onxeo's international development plan, the company has conducted this Phase III trial in 11 countries (Europe, USA, MENA*).

To date, 390 patients have been randomized, with about 260 patients in the Livatag® treatment group and 130 in the the comparative group (best standard of care). The completion of patient randomization is an important milestone that confirms the expected timeline of issuing the preliminary efficacy outcomes of the study mid-2017.

"Completion of enrollment in ReLive marks a significant milestone for the HCC community. This is a major step forward in the development of Livatag® as a new therapeutic option in a pathology for which there is a strong need for new treatment. It is also a major achievement for Onxeo, which demonstrates its ability to complete a large international Phase III trial and marks a major value creation catalyst, in line with the expected publication of preliminary results in mid-2017," concludes Judith Greciet, Chief Executive Officer of Onxeo. *"On behalf of Onxeo, we would like to extend our warmest thanks to the investigators and coordinators of the clinical sites for their active participation that enabled us to carry out this trial".*

From a safety standpoint, nine DSMB** reviews of the ReLive study have already been conducted over the course of the study. During these reviews, the experts did not identify unexpected adverse effects or signals and unanimously recommended each time the continuation of the trial without modification. These repeated positive recommendations - based on data from the administration of almost 1,000 Livatag® infusions - seem to indicate an adequate safety profile of the drug to date. These findings are important because they reflect the likelihood that this profile will be acceptable to both physicians and regulators.

Livatag®, an innovative therapeutic approach to hepatocellular carcinoma

Livatag® is a nanoparticle formulation of doxorubicin, developed using Onxeo's proprietary Transdrug™ technology designed to facilitate the penetration of the drug into the tumor cell and increase the target DNA exposure to the drug, thereby bypassing the mechanisms of multi-drug resistance developed by tumor cells. By specifically accumulating in the liver cells and overcoming resistance to doxorubicin, Livatag® represents a potentially significant breakthrough in the treatment of hepatocellular carcinoma.

Hepatocellular Carcinoma, an aggressive form of primary liver cancer

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 6th most common cancer in terms of incidence (782,000 new cases worldwide each year, 5.6% of all new cancer cases) with the 2nd highest mortality rate (95% lethality) after lung cancer. The major risk factors are infection by hepatitis viruses (B and C), overconsumption of alcohol and metabolic diseases, especiallynon-alcoholic steato-hepatitis, a growing cause of cirrhosis and HCC.

** MENA: Middle-East & North Africa*
*** DSMB: Data Safety and Monitoring Board*

About Onxeo

Onxeo is a biotechology company developing innovative drugs for the treatment of orphan diseases in oncology, driven by high therapeutic demand in one of the fastest growing segments of the pharmaceutical industry. Onxeo's objective is to become a major international player in the field of rare cancers. Its growth strategy is founded on the development of innovative, effective, and safe drugs based on breakthrough technologies that can make a real difference in the treatment of orphan oncology diseases and considerably improve the quality of life of patients affected by rare and aggressive cancers. Onxeo's comprehensive portfolio features a broad orphan oncology pipeline, with four independent programs in various stages of clinical development, including Onxeo's first approved orphan oncology drug, Beleodaq®. 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's orphan oncology products 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 US 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 US, 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 I 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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