Onxeo Announces Promising Results from Preclinical Studies of Livatag® in Pancreatic Cancer

Paris (France), Copenhagen (Denmark), November 4, 2016 – 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 encouraging results from a series of preclinical studies evaluating Livatag® interest for pancreatic cancer.

Livatag® (doxorubicine Transdrug™, a nanoparticle formulation of doxorubicin) is currently being evaluated clinically as a monotherapy in a Phase III ReLive trial for second-line advanced hepatocellular carcinoma (HCC). More than 90% of expected patients have been randomized and study preliminary data are expected mid-2017.

In parallel, the company has initiated an exploratory preclinical evaluation program of Livatag® for new indications, such as pancreatic cancer, which is part of Onxeo's strategic efforts to capitalize on the specific benefits of Livatag® (doxorubicin Transdrug™, a nanoparticle formulation of doxorubicin) and the acquired knowledge of its mechanism of action to provide potential new therapeutic options for patients with unmet medical needs and further generate additional value for shareholders with this promising compound.

A first set of preclinical studies has been performed and confirmed several key advantages of Livatag® versus free doxorubicin:

- Livatag® demonstrated on average a two-fold increase in potency compared to free doxorubicin when tested on a range of pancreatic cell lines. Additionally, Livatag® significantly extended the plasma life-time of the active ingredient doxorubicin compared with conventional formulation of free doxorubicin.
- In murine pancreatic cancer models where free doxorubicin had limited effect, Livatag® exhibited good, dose-dependent efficacy. Similar to results previously shown in HCC, these preclinical studies showed that Livatag® has good exposure in pancreatic tumors without increasing exposure in other vital organs such as heart and lungs, when compared to free doxorubicin.
- Pancreatic cancer is a stromal tumor, which is known in literature to exhibit preferential uptake of particles, and this is likely a contributory factor to the increased potency observed with Livatag® compared to free doxorubicin in the pancreatic cancer animal models.
- This study in murine orthotopic pancreatic cancer models also demonstrated, similarly to previous observations with Livatag® in HCC models, that Livatag® in combination with checkpoint inhibitors resulted in supra-additive efficacy.

Following the initial mechanisitic and pharmacokinetic/pharmacodynamic data, the company intiated a study plan to assess the effect of Livatag® in comparison with current standard treatments, either in monotherapy or in combination to assess how these first data might translate into the clinical setting to validate the potential interest of Livatag® in this indication. In a mouse syngeneic pancreatic cancer model, Livatag® as a monotherapy proved to be as effective or more effective than current therapies, including chemotherapies gemcitabine, paclitaxel, and erlotinib. In addition, the study demonstrated that Livatag® combines well with these three therapies, showing good tolerance and exhibiting supra-additive efficacy in all combinations compared to each agent alone.

Graham Dixon, PhD, Chief Scientific Officer of Onxeo, commented, *"The results from these preclinical studies of Livatag® in pancreatic cancer have generated promising potential for the drug in this indication. These exploratory data on the mechanistic rational of Livatag® for pancreatic cancer, supported by the outcomes in comparison with clinical standards, provides a strong rationale to fully explore the opportunity to further develop Livatag® in this indication. We are also highly encouraged by the supra-additive effects observed in combination with checkpoint inhibitors in these animal models, which we will further explore."*

About Onxeo

Onxeo is a biopharmaceutical company specializing in the development of innovative drugs for the treatment of orphan diseases, in particular 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); Livatag is also under exploratory preclinical development to assess interest of its combination with other anti cancer agents.
- **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 a oral formulation is under development.
- **AsiDNA**: first-in-class siDNA (signal-interfering DNA) which has successfully undergone a proof-of-concept Phase I trial in metastatic melanoma via local administration
- **Validive®** (Clonidine Lauriad®): Positive final results from a Phase II trial in head and neck cancer patients with severe oral mucositis

In addition, Onxeo has successfully developed and registered two non-cancer products, which are currently being commercialized in the U.S. and Europe.

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