Onxeo Announces First Outcomes of Livatag® Preclinical Plan

- *Livatag® Nanoformulation Leads to Increased Exposure and Preferential Affinity for Liver, Supporting Current Relive Phase III Study Rationale*
- *Demonstrates Enhanced Effect in Combination with Immunotherapy*

Paris (France), Copenhagen (Denmark), September 12, 2016 – Onxeo S.A. (Euronext Paris, Nasdaq Copenhagen: ONXEO), an innovative company specializing in the development of orphan oncology therapeutics, today announced data from 2 in vivo studies of the Livatag® preclinical plan confirming that the nanoparticle formulation meets the pharmacological requirements for a hepatocellular carcinoma (HCC) treatment and, furthermore, that the combination of Livatag® with immunotherapy produces an enhanced efficacy effect, validating Onxeo's comprehensive strategy to explore further potential indications for its key product candidate.

The studies were performed in an orthotopic (implantation of tumor cells into the liver of mice) HCC model in immune-competent mice. Orthotopic models are considered relevant to the clinical situation and a good predictor of drug efficacy. Results demonstrate that Livatag® (doxorubicin Transdrug™) generates a 12-fold increase in exposure in the tumor tissue within the liver compared to free doxorubicin, without increasing the drug's exposure in the heart or other vital organs. These findings complete, and go beyond, data from a mechanistic study of Livatag® previously reported at the 2016 American Association for Cancer Research (AACR) Annual Meeting that revealed that the distribution of Livatag® nanoparticles in healthy liver was approximately six times higher compared to free doxorubicin, which indicate that Livatag® nanoparticles have a preferential affinity for and are able to target the liver and more particularly, the liver tumor tissue.

As part of this program, Onxeo has also been exploring the potential of Livatag® when administered with emerging immuno-oncology agents of various classes, such as promising PD-1 and CTLA-4 checkpoint inhibitors currently in development. A current study demonstrates that Livatag® produces an enhanced effect in tumor response (reduction in tumor volume) when given in combination with immuno-oncology agents in the orthotopic HCC model. Specifically, Livatag® co-administration with antibodies is associated with a positive increase in circulating T-cell populations, which is consistent with the observed reduction in tumor volume.

Graham Dixon, PhD, Chief Scientific Officer at Onxeo, commented, "*These preclinical findings are important because they reinforce our previous research showing that Livatag® Transdrug™ technology dramatically improves the drug's exposure in liver cancer tissue, demonstrating high affinity for the liver, without increasing exposure in other organs. These data validate the interest of Livatag® as a potential new therapeutic option for HCC treatment. Besides, we are pleased to share the first data demonstrating*

an enhanced anti-cancer response generated by Livatag® in combination with immuno-oncology agents in murine HCC models. Results from this study will enable us to extend the value of one of our lead assets and add to the growing value of Onxeo's pipeline. In addition to sharing results from the Phase III ReLive trial next year, we look forward to continued evaluation of Livatag® in combination with immunotherapy agents and delivering a plan to advance Livatag® in the clinic in additional indications."

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 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. 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

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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Contact:
Judith Greciet, CEO
Nicolas Fellmann, CFO
investors@onxeo.com
+33 1 45 58 76 00

Caroline Carmagnol /Florence Portejoie – Alize RP (France)
onxeo@alizerp.com
+33 6 64 18 99 59 / +33 6 47 38 90 04

Kirsten Thomas / Lee Roth – The Ruth Group (U.S.)
kthomas@theruthgroup.com / lroth@theruthgroup.com
+1 508 280 6592 / +1 646 536 7012