Onxeo Reports Third Quarter 2016 Financial Information and Provides Business Update

- *Clinical development plan in line with objectives*
- *Livatag® on track to deliver its next important milestone with 90% of patients randomized*
- *€12.5m capital increase successfully completed*

Paris (France), Copenhagen (Denmark), October 25, 2016 – Onxeo S.A. (Euronext Paris, Nasdaq Copenhagen: ONXEO), an innovative company specialized in the development of orphan oncology therapeutics, today published its consolidated financials for the period ending September 30, 2016 and provided an update on major milestones achieved during the third quarter of 2016.

"The third quarter of 2016 was particularly eventful and productive for Onxeo. We made remarkable progress in terms of advancing our three key portfolio products as well as on the business development front. We announced results from two important preclinical studies, the first of which reinforces the rationale for developing Livatag® as a potential new therapeutic option for HCC. Regarding the Livatag "ReLive" study, we are well on track to finalize the recruitment of patients in the near term, allowing the release of preliminary results in mid-2017 as planned. Data from another preclinical study confirmed the potential benefits of using AsiDNA™ in combination with PARP inhibitors such as olaparib. This summer, we signed an exclusive licensing agreement with Pint Pharma for Beleodaq® in South America, further expanding our product's commercial potential. Lastly, our successful capital increase executed at the end of the third quarter has enabled us to increase our cash runway and strengthen our institutional shareholder base, including a number of US-based, specialized investors. We are well-equipped to address the opportunities expected in the coming months, as we work to deliver innovative therapeutic options that patients critically need, while creating value for our shareholders," commented Judith Greciet, CEO of Onxeo.

Continued advancement on key assets

- **Comprehensive preclinical and clinical work strengthening the products' potential**

Onxeo has progressed in the development of its key compound, **Livatag®** (doxorubicin nanoformulation in Phase III trial for treatment of hepatocellular carcinoma). With more than 90% of the patients randomized as of September 30, 2016, the company is on track to deliver the preliminary results of the ReLive Phase III clinical study in mid-2017, in line with its development plan.

In an effort to expand the application of Livatag® into other indications, Onxeo has also announced the first outcomes of its Livatag® preclinical program, demonstrating enhanced efficacy effect in combination with immunotherapy, which validates its broader strategy for the product. Data from two *in vivo* studies have also confirmed the increased exposure and preferential affinity for the liver, supporting Onxeo's current ReLive Phase III study rationale.

Onxeo has also made significant progress on **Beleodaq®**, its pan-HDAC inhibitor already approved for PTCL (peripheral T-cell lymphoma). The company has started an initiative to develop an oral formulation of the compound, which would give a clear competitive advantage as well as expand the product's potential application to indications for which such an oral formulation is appropriate. This development is on track, with prototypes designed and improved bioavailibity shown in an animal pharmacokinetic study.

Moreover, the company recently signed a promising new collaboration with the Royal College of Surgeons in Ireland (RCSI) for a research program on Beleodaq® conjugate molecules, to improve product lifetime and stability properties, ultimately aiming to generate new patent opportunities.

- **Active preparation for the clinical development of first-in-class product AsiDNA™**

Since the AsiDNA™ acquisition, the Company has undertaken significant efforts to optimize the manufacturing process in terms of cost and duration, and is on track to manufacture its first clinical batch by the end of 2016, allowing for the initiation of a Phase I trial planned for 2017, after appropriate regulatory toxicologic assay.

The Company's first objective is to show AsiDNA™ activity when administered via the IV route, which would dramatically expand the potential of this compound. In parallel, preclinical research demonstrating the synergistic effect of Onxeo's signal-interfering DNA product candidate in combination with various PARP (PolyADP-Ribose Polymerase) inhibitors has been published, confirming the interest of AsiDNA™ compared to PARP inhibitors alone and the interest of the combination of these two DNA repair inhibitors.

Solid progress in business development and intellectual property

In the third quarter, Onxeo has strengthened its AsiDNA™ intellectual property portfolio in the US with a new patent valid until 2031, confirming the innovative nature of the science behind its signal-interfering DNA product.

The company was also actively engaged in key operational and business development initiatives and achieved an important business development milestone, signing an exclusive license agreement with Pint Pharma for the commercialization of Beleodaq® (belinostat) for PTCL in seven major South American countries.

Q3 revenue growth

Revenues for the third quarter of 2016 amounted to €1.23 million compared to €1.1 million in the third quarter of 2015 (+8%).

- €0.8 million of recurring revenues corresponding to product supplies to commercial partners and royalties on partners' sales
- €0.4 million of non-recurring revenues, relating to the recognition under IFRS of upfront payments on certain licensing agreements

Over the first 9 months of the year, total revenues stood at €3.1 million, out of which €2.6 million were recurring revenues vs. €2.0 million in 2015 (+30%).

Long-term visibility reinforced with a successful €12.5 million capital increase

In early October, Onxeo successfully completed a capital increase of 5,434,783 new ordinary shares, raising gross proceeds of €12.5 million in a Private Placement. This capital increase strengthens and diversifies Onxeo's shareholder base with the addition of prominent US-based healthcare institutional investors.

Proceeds from the capital increase, received on October 5, add to the €22.4 million consolidated cash balance at the end of September 2016, which extends Onxeo's cash runway until Q2 2018. This capital

will allow the company to pursue and accelerate the ongoing development of its pipeline assets, including the AsiDNA™ and Livatag® programs, as well as advance key preclinical programs, such as the combination therapy studies for AsiDNA™, Livatag®, and Beleodaq®.

Key near- and mid-term milestones

- Livatag®:
  - Preclinical combination plan
  - Next DSMB for Phase III trial: Q4 2016
  - Preliminary Phase III trial results: expected mid-2017
- AsiDNA™:
  - Phase I initiation (monotherapy systemic) now expected in 2017, based on current CMC progress
- Beleodaq®:
  - New oral formulation validated, ready to enter clinic: Q3 2017
  - Preclinical combination study results: end of 2016 and onwards
  - 1st-line PTCL Phase III initiation: end of 2016

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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