THE ORPHAN ONCOLOGY INNOVATOR

**PRESS RELEASE**

# *Onxeo Announces Top-Line Results from ReLive Phase III Study of Livatag® in Advanced Hepatocellular Carcinoma*

**Paris (France), September 11, 2017 – 8:00 pm CEST**– Onxeo S.A. (Euronext Paris, NASDAQ Copenhagen: ONXEO), ("**Onxeo**" or the "**Company**"), a clinical-stage biotechnology company specializing in the development of innovative drugs for the treatment of orphan diseases, in particular in oncology, today announced top line results from the phase III ReLive trial of Livatag® (doxorubicine Transdrug™) in adult patients with unresectable hepatocellular carcinoma (HCC), intolerant to sorafenib or having progressed after a systemic therapy including sorafenib, when compared to best standard of care. The study did not meet its primary endpoint of improving survival over the comparative group.

The major reason is an unexpected high survival in the comparative group. Indeed, the study was not placebo controlled and patients in the comparative group could receive other anticancer agents (including oxaliplatin, gemcitabine or tyrosine kinase inhibitors) which might explain the high survival rate of the control arm. Livatag®, as single agent, showed a similar effect as the one observed in that comparative group with active treatments. There was no difference in efficacy between the two arms (Livatag 20mg/m² and 30mg/m²).

The overall safety and tolerability profile of Livatag® in ReLive was favorable with a fully manageable toxicity profile in both groups of Livatag (20mg/m² and 30mg/m²) including in those patients who underwent the longest treatment periods, over one year. The overall tolerability was comparable to the one observed in the comparative group.

*"The Relive study did not meet its primary endpoint, partly due to the high survival rate in the control arm, which was unprecedented except in the most recent phase III negative trial post Sorafenib in HCC. However, Livatag tends to show a similar level of efficacy as recently reported for regorafenib in second line, in a well preserved liver function population (Child– Pugh A), although both drugs cannot be compared due to the lack of assessment of both drugs in the same trial."* commented Philippe Merle, MD, Professor in Hepatology (La Croix Rousse Hospital, Lyon, France) and Coordinating Investigator of the ReLive study. *"We want to thank all the investigators who have supported the completion of this large phase III trial as well as the patients and their families, and we are confident that they benefited in participating in the Relive study."*

The monitoring of the patients still enrolled in the study will continue to completion expected in H1 2019.

The analysis of predefined subgroups is ongoing and the main results from the ReLive study will be presented on at the upcoming 11th Annual Conference of the International Liver Cancer Association in Seoul, South Korea (ILCA 2017 - ilca2017.org).

*"Once the Relive data are fully analyzed, we will reinitiate licensing discussion with potential partners based on key study outcomes to define the best path forward",* said Judith Greciet, Chief Executive Officer of Onxeo. "*As already announced, Onxeo will continue to advance its diversified pipeline of innovative assets in oncology. Refocusing our R&D activities on AsiDNA™ and Beleodaq® should extend our financial visibility until early 2020."*

## About 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, especially non-alcoholic steatohepatitis (NASH), a growing cause of cirrhosis and HCC.

## About ReLive Phase III trial

This international, multicenter, randomized, comparative Phase 3 trial was conducted in 11 countries (Europe, USA, and MENA) at 70 centers and enrolled 397 adult patients with unresectable hepatocellular carcinoma (HCC), intolerant to sorafenib or having progressed after a systemic therapy including sorafenib. Patients were randomized to receive Livatag® administered intravenously for 6 hours every 4 weeks (n=263) or best standard of care, i.e. any cancer therapy chosen by the physician except sorafenib (n=134). Treatment was continued until disease progression or unacceptable toxicity. The monitoring of the patients enrolled in the study will continue to completion, expected Q1 2019.

## Upcoming events

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| **September 16, 2017** | 11th meeting of the International Liver Cancer Association<br>Oral Presentation of ReLive Results | Seoul, South Korea |
| **October 2nd, 2017** | French Society of Financial Analysts meeting<br>ReLive Results & Strategic Update | Paris, France |
| **October 4-5, 2017** | Large & MidCap Forum | Paris, France |
| **October 19, 2017** | Portzamparc Biotech Symposium | Paris, France |
| **October 26, 2017** | Q3 results and business update | |

## About Onxeo

Onxeo is a biotechnology 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 or resistant cancers. Its growth strategy is to develop innovative, effective, and safe drugs based on breakthrough technologies that can make a real difference in patients' lives, by acquiring or in-licensing first-in-class or unique compounds at an early stage and bringing them through translational research and proof of concept clinical development up to value-creating inflexion points. Onxeo's orphan oncology pipeline comprises products in several on-going preclinical and clinical programs, alone or in combination for various cancer indications.

- **Livatag®** is a nanoparticle formulation of the chemotherapy 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.
- **AsiDNA™:** a first-in-class siDNA (signal-interfering DNA) candidate which has successfully undergone a proof-of-concept Phase I trial via local administration in metastatic melanoma. Recent positive preclinical proof-of-concept results confirmed AsiDNA™ activity via systemic administration in a murine model of triple negative breast cancer (TNBC). The Company now prepares a phase I trial via systemic (intravenous) administration, expected to be submitted to the regulatory authorities by the end of 2017.
- **Beleodaq®** (belinostat): a HDAC inhibitor, conditionally 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 anti-cancer agents is also in ongoing development in 1st line treatment for patients with PTCL (BelCHOP) and in solid tumors.

Both AsiDNA™ via intravenous administration and belinostat (notably its oral formulation) are currently undergoing intense preclinical activities, alone and in combination, to determine the best combinations, indications and clinical pathways, with already promising data.

Additional preclinical results will be announced in the coming weeks and first clinical studies will be submitted to regulatory authorities before year-end 2017, as previously announced.

The Company is headquartered in Paris, France with offices in Copenhagen and in New York, and has approximately 60 employees. Onxeo is listed on Euronext in Paris, France and Nasdaq Copenhagen, Denmark (Ticker: ONXEO, ISIN Code: FR0010095596).

Learn more by visiting www.onxeo.com

**Forward looking statements**

This communication expressly or implicitly contains certain forward-looking statements concerning Onxeo and its business. Such statements involve certain known and unknown risks, uncertainties and other factors, which could cause the actual results, financial condition, performance or achievements of Onxeo to be materially different from any future results, performance or achievements expressed or implied by such forward-looking statements. Onxeo is providing this communication as of this date and does not undertake to update any forward-looking statements contained herein as a result of new information, future events or otherwise. For a discussion of risks and uncertainties which could cause actual results, financial condition, performance or achievements of Onxeo to differ from those contained in the forward-looking statements, please refer to the section 5.5.1.4 "Risk Factors" ("*Facteurs de Risque*") of the 2016 reference document filed with the *Autorité des marchés financiers* on April 24, 2017 under number D.17-0423, which is available on the *Autorité des marchés financiers* website (www.amf-france.org) or on the Company's website (www.onxeo.com).

**Onxeo**
Valerie Leroy
Investor Relations
investors@onxeo.com
+33 1 45 58 76 00

**Media Relations**
Caroline Carmagnol / Alize RP
alize-onxeo@alizerp.com
+33 6 64 18 99 59

**Investor Relations / Strategic Communications**
Dušan Orešanský / Emmanuel Huynh
NewCap
onxeo@newcap.eu
+33 1 44 71 94 92

**Investor Relations US**
Brian Ritchie
LifeSci Advisors
britchie@lifesciadvisors.com
+1 212 915 2578