THE ORPHAN ONCOLOGY INNOVATOR

PRESS RELEASE

Onxeo to Present Data Supporting Three Key Orphan Oncology Assets at AACR Annual Meeting

Preclinical studies of AsiDNA™, Livatag® and Beleodaq®

Paris (France), March 21, 2017 – 6 pm CET – Onxeo S.A. (Euronext Paris, NASDAQ Copenhagen: ONXEO), a biotechnology company specializing in the development of innovative drugs for the treatment of orphan diseases, in particular in oncology, announced today the presentation of data from three studies supporting the company's primary drug candidates in oncology, AsiDNA™, Livatag® and Beleodaq® in poster sessions at the upcoming American Association for Cancer Research (AACR) Annual Meeting, one of the most prestigious meetings on preclinical cancer research, being held April 1-5, 2017 in Washington, D.C.

Françoise Bono, PhD, Chief Scientific Officer, commented, "*The various data we will be presenting at the prominent AACR oncology meeting demonstrates our commitment to further explore and advance our three core pipeline assets. These data support delayed tumor growth with the combination of our signal-interfering molecule AsiDNA™ and PARP inhibitors, the ability of Livatag® to reverse chemo-resistance compared to free doxorubicin and increased anti-tumor response through the combination of our marketed drug Beleodaq® with immune checkpoint inhibitors. The results provide a strong rationale for the continued development of each product candidate, and this validation is directly in-line with our mission of identifying and developing innovative approaches to fight some of the most aggressive cancer indications. We look forward to presenting these findings at the conference.*"

Details of the sessions on April 3 and 4 include:

Abstract 1110 / Poster 3 **– AsiDNA™ induce tumor sensitivity to PARP inhibitors in homologous recombination proficient breast cancer**

Session:	PO.ET03.01 - DNA Repair
Date:	Monday, April 3
Time:	8:00 a.m. – 12:00 p.m. ET
Location:	Section 3

The study highlights the therapeutic interest of combining Onxeo's lead signal-interfering DNA product candidate AsiDNA™ and PARP (PolyADP-Ribose Polymerase) inhibitors. This combination significantly decreases tumor growth independent of genetic mutations, in contrast to the antitumoral efficacy of PARP inhibitors alone, which are only effective against tumors bearing mutations on genes coding for proteins involved in homologous recombination (HR) pathway. In a preclinical murine tumor model, while the PARP inhibitor olaparib failed to prevent tumor growth and AsiDNA partially delayed this growth, the combination of olaparib and AsiDNA demonstrated synergistic antitumor efficacy.

Most interestingly, no resistant clones to AsiDNA™ appeared, suggesting sustained clinical efficacy, unlike most targeted therapies.

Abstract 3076 / Poster 14 **– A novel nanoparticle formulation of doxorubicin is clearly differentiated from free doxorubicin in overcoming resistance mechanisms in chemo-resistant tumors**

Session: PO.ET02.04 - Determinants of Drug Sensitivity and Resistance
Date: Tuesday, April 4
Time: 8:00 a.m. – 12:00 p.m. ET
Location: Section 2

The study demonstrates the therapeutic potential of Livatag®, doxorubicin loaded nanoparticles, to reverse chemo-resistance compared to free doxorubicin in hepatocellular carcinoma (HCC, primary liver cancer), pancreatic cancer and sarcoma models. Livatag® showed a dose-dependent inhibition of cell proliferation in all tested resistant cancer cell lines with superior activity compared to free doxorubicin and other tested drugs. Moreover, in contrast to free doxorubicin, Livatag® showed consistent anti-proliferative activity in the absence or presence of inhibitors of efflux pumps and autophagy. In a range of in vivo models, Livatag® was preferentially taken up by the tumor tissue and significantly reduced tumor growth when compared with free doxorubicin, with at least equivalent reduction in tumor growth compared to currently approved treatments. Furthermore, Livatag® administered in combination with current treatments significantly increased the inhibitory effect of each drug without additional toxicity.

In this study, Livatag® is clearly differentiated from free doxorubicin in its ability to overcome resistance mechanisms linked to efflux and autophagy, and its superior bio-distribution profile, both of which result in significantly enhanced activity on chemotherapy-resistant tumors.

These new results collectively support the strong rationale behind the ongoing Phase III ReLive clinical trial comparing Livatag® to the best standard of care in patients with advanced HCC. Recruitment in this trial is complete, and preliminary results are expected mid-year.

Abstract 1059 / Poster 12 **– Enhanced anti-tumor efficacy of a checkpoint inhibitor in combination with the HDAC inhibitor belinostat in a murine hepatocellular carcinoma preclinical model**

Session: PO.ET02.02 - Combination Strategies: Novel Agents and Standard Therapies
Date: Monday, April 3
Time: 8:00 a.m. – 12:00 p.m. ET
Location: Section 1

Results demonstrated that Beleodaq® (belinostat) improved anti-tumor therapeutic response induced by the checkpoint inhibitor, anti-CTLA4, showing significantly superior tumor growth inhibition compared to control groups. Importantly, treatment with the combination resulted in complete cessation of tumor growth in all mice during the belinostat treatment period. Mechanistic studies showed that Beleodaq® (belinostat) induces an increase in the production of interleukins (proteins involved in the signaling and regulation of immune response) by activated T-lymphocytes, and a concomitant decrease in the regulatory T cells (immunosuppressive cells) in the spleens of treated animals. These results provide strong rationale for using belinostat, which is approved in the U.S. for the treatment of second-line peripheral T cell lymphoma (PTCL), in combination with checkpoint inhibitors to reinforce therapeutic response. Currently, only 20 to 40% of patients respond to checkpoint inhibitors alone. In parallel, a new oral formulation of belinostat will allow potential use in multiple clinical situations.

Further studies are ongoing in order to fully characterize this finding and to facilitate translation into patients.

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 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 or resistant cancers.

Onxeo's comprehensive portfolio features a broad orphan oncology pipeline, with 3 major products in several on-going preclinical and clinical programs, alone or in combination for various cancer indications.

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

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