

Onxeo to Present Results of Two Studies Highlighting Potential of AsiDNA™ as Anti-Cancer Treatment at 2018 AACR Annual Meeting

Paris (France), March 15th, 2018 – 7:00 am CET - Onxeo S.A. (Euronext Paris, NASDAQ Copenhagen: ONXEO FR0010095596), a biotechnology company specializing in the development of innovative drugs in oncology, and notably rare and resistant forms of cancer, today announced the presentation of two preclinical study abstracts highlighting AsiDNA™, the Company's first-in-class DNA break repair inhibitor candidate, at the upcoming American Association for Cancer Research (AACR) Annual Meeting being held April 14-18, 2018 in Chicago, Illinois.

"We believe AsiDNA™, our lead product candidate, holds significant promise as a treatment for solid tumors," said Françoise Bono, PhD, Chief Scientific Officer. "We have achieved two significant outcomes related to our preclinical plan for which data will be presented at AACR. Firstly, we have shown the strong therapeutic potential of AsiDNA™ in combination with an HDAC inhibitor and, secondly, we have demonstrated a unique and highly compelling characteristic of AsiDNA™ that could provide the opportunity to use the product as maintenance therapy. Indeed, the first study shows the highly synergistic antitumor activity of the combination of AsiDNA™ with belinostat, Onxeo's HDAC inhibitor, in several tumor models. In the second study, long-term treatment with AsiDNA™ has shown to lead to an increased sensitivity of tumor cells. This is a unique feature in the setting of cancer treatment that could lead to a new development opportunity for AsiDNA™ in maintenance, to prevent treatment resistance."

Details of the sessions include:

[Abstract # / Poster #](#) -- AsiDNA™ and HDAC inhibitors: a cross-potential™ team working to kill tumor cells

Session: Modulation of DNA Damage and Repair

Date: Monday Apr 16, 2018

Time: 1:00 PM - 5:00 PM

Place: McCormick Place South, Exhibit Hall A, Poster Section 37

This study evaluated the antitumor efficacy of the combination of HDAC inhibitors with Onxeo's DNA damage repair (DDR) inhibitor, AsiDNA™. The results of this study demonstrated that belinostat treatment induces DNA break accumulation and genetic instability in tumor cells specifically. In addition, AsiDNA™ induced a high potentiation of belinostat activity on its targets. This mechanism-based cross-potential between AsiDNA™ and belinostat resulted in highly synergistic antitumor efficacy in different tumor models. Moreover, while repeated treatments induced the emergence of resistance to belinostat alone, this resistance did not appear in the combination with AsiDNA™, indicating the potential of the combination to prevent tumor escape from treatment.

These results strongly support the rationale to investigate the in-vivo activity of this novel synergistic combination in different tumor types, which can then be followed by clinical evaluation. Belinostat previously received U.S. Food and Drug Administration (FDA) conditional approval, and AsiDNA™ will begin a phase I with a systemic administration shortly.

**Abstract # / Poster # -- Evolution of tumor cells under Dbait treatment results in “autosensitization”**

Session: Experimental and Molecular Therapeutics

Date: Monday Apr 16, 2018

Time: 1:00 PM - 5:00 PM

Place: McCormick Place South, Exhibit Hall A, Poster Section 38

This study was conducted in collaboration with Marie Dutreix, PhD, of the Institut Curie in Paris, France. AsiDNA™ is a new and unique approach to tumor DNA repair inhibitor (Dbait approach) that activates the enzymes involved in DNA damage signaling. In this preclinical study, Onxeo evaluated how such agonist activity would limit resistance to treatment.

The results demonstrated that long-term repeated treatments with AsiDNA™ led to increased sensitivity of tumor cells to the molecule itself, contrary to what was observed with some targeted therapies such as olaparib, imatinib and 6-thioguanine. The six tumor cell lines tested developed increased sensitivity to AsiDNA™ and no resistance after cyclic treatments. Non-tumoral cells were not affected by repeated treatments. The acquired sensitivity of the treated tumor cell populations was preserved for one month following the conclusion of treatment.

These results indicate autosensitization took place during treatment, a characteristic not previously observed in anticancer therapy. This unique property could provide the opportunity for AsiDNA™ to be used in maintenance therapy due to its capacity to prevent the development of acquired resistance during treatment, and should be further confirmed in the near future.

Onxeo has filed a priority patent application claiming the use of maintenance therapy with AsiDNA™ based on its newly-identified property.

About Onxeo

Onxeo (Euronext Paris, NASDAQ Copenhagen: ONXEO) is a French biotechnology company developing innovative oncology drugs based on DNA-targeting and epigenetics, two of the most sought-after mechanisms of action in cancer treatment today. The Company is focused on bringing early-stage first-in-class or disruptive compounds (proprietary, acquired or in-licensed) from translational research to clinical proof-of-concept, a value-creating inflection point appealing to potential partners.

Onxeo's R&D pipeline includes **belinostat**, an HDAC inhibitor (epigenetics) currently being developed in oral form to be used in combination with other anti-cancer agents for liquid or solid tumors. Belinostat is already conditionally FDA-approved in the US as a 2nd line treatment for patients with peripheral T cell lymphoma and marketed in the US by Onxeo's partner, Spectrum Pharmaceuticals, under the name Beleodaq® (belinostat IV form).

Onxeo is also developing **AsiDNA™**, a first-in-class DNA break repair inhibitor based on a unique decoy mechanism. AsiDNA™ has already successfully completed a Phase I trial in metastatic melanoma via local administration, and is currently being developed for systemic (IV) administration in solid tumors.

AsiDNA™ is the first compound generated from **platON™**, the Company's proprietary chemistry platform of decoy oligonucleotides based on three components, a sequence of double strand oligonucleotides, a linker and a cellular uptake facilitator. PlatON™ will continue to generate new compounds that will broaden Onxeo's pipeline.

For further information, please visit 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).

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