

## ***Onxeo to Present Final Results of DRIIV-1 Phase 1 Study of AsiDNA™ in Advanced Solid Tumors at AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics***

### **Positive Safety and Activity Results from DRIIV-1 Led to Initiation of Ongoing DRIIV-1b Phase 1b Study of AsiDNA™ in Combination with Chemotherapy**

Paris (France), October 15, 2019 – 6:00 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 targeting tumor DNA Damage Response (DDR) in oncology, in particular against rare or resistant cancers, today announced that the Company will present the final results of its DRIIV-1 Phase 1 study of AsiDNA™, the Company’s first-in-class DDR inhibitor, in a poster session on October 27, 2019, during the AACR-NCI-EORTC International Conference on Molecular Targets and Cancer Therapeutics in Boston.

**Olivier de Beaumont, Medical Director of Onxeo, commented:** *“DRIIV-1 demonstrated AsiDNA’s favorable safety profile and validated its mechanism of action in patients’ tumor cells through the activation of its biological targets. Most importantly, the optimal active dose of AsiDNA™ of 600 mg was determined and is currently being utilized in our ongoing DRIIV-1b study, which is evaluating AsiDNA™ in combination with chemotherapy. DRIIV-1 was the foundation of our clinical development strategy for AsiDNA™ via intravenous administration and we look forward to presenting and discussing the compelling results of this important study, as well as reviewing our anticipated next steps, with the international oncology community.”*

The poster, titled ***“Phase I dose escalation study evaluating the safety, pharmacokinetics (PK) and pharmacodynamics (PD) of AsiDNA, a first-in-class DNA Repair Inhibitor, administered intravenously (IV) in patients with advanced solid tumors,”*** will be presented by Professor Christophe Le Tourneau, principal investigator of the study and Head of the Department of Drug Development and Innovation at the Curie Institute (Paris, France).

➤ <b>Session Title</b>	Clinical Trials
➤ <b>Session Date</b>	Sunday, October 27
➤ <b>Session Start Time</b>	12:30
➤ <b>Session End Time</b>	16:00
➤ <b>Location</b>	Hall D, Hynes Convention Center
➤ <b>Permanent Abstract Number</b>	A076

The primary objective of this open label, dose escalation study, was to establish dose-limiting toxicities and identify the maximum tolerated dose of AsiDNA™ administered intravenously (IV). Other objectives included evaluating the product candidate’s safety profile, pharmacokinetic and pharmacodynamic parameters and preliminary efficacy data. Twenty-two patients with advanced solid tumors having failed previous anticancer therapies received a loading dose of AsiDNA™ for three consecutive days, followed by a one-hour IV-infusion once per week in 21 day cycles. In each subsequent cycle, AsiDNA™ was given weekly and administered until disease progression, unacceptable toxicity or patient’s decision.



Biological activity was evidenced by the increase of  $\gamma$ H2AX and pHSP90, two intratumoral biomarkers of DNA-PK, one of the DDR proteins targeted by AsiDNA™. The product candidate's favorable safety profile was confirmed, with 90% of all product-related adverse events being non-specific grade 1 and 2 events. The maximum tolerated dose was not reached. The 600 mg dose has been identified as the optimal biological dose for further development due to the favorable safety and pharmacokinetic profile, as well as robust target engagement, demonstrated at this dosage level. Disease stabilization was achieved in two patients with advanced colorectal cancer.

#### Upcoming events

▪ October 23-24, 2019	Conférence Galien MedStart'Up	New York, NJ, USA
▪ October 26-30 2019	Conférence 'Molecular Targets & Cancer Therapeutics' de l'ACR-NCI-EORTC	Boston, MA, USA
▪ November 6, 2019	Évènement Direct Dirigeants	Paris, France
▪ November 12-13, 2019	Bryan, Garnier & Co European Healthcare Conference	Paris, France
▪ November 12-15, 2019	Tides Europe 2019	Amsterdam, Holland
▪ January 29-31, 2020	DNA Damage Response Therapeutics Summit 2019	Boston, MA, USA

#### About Onxeo

**Onxeo** (Euronext Paris, NASDAQ Copenhagen: ONXEO) is a clinical-stage biotechnology company developing innovative oncology drugs targeting tumor DNA-binding functions through unique mechanisms of action in the sought-after field of DNA Damage Response (DDR). The Company is focused on bringing early-stage first-in-class or disruptive compounds from translational research to clinical proof-of-concept, a value-creating inflection point appealing to potential partners.

**platON™** is Onxeo's proprietary chemistry platform of oligonucleotides acting as decoy agonists, which generates new innovative compounds and broaden the Company's product pipeline.

**AsiDNA™**, the first compound from platON™, is a first-in-class, highly differentiated DNA Damage Response (DDR) inhibitor based on a decoy and agonist mechanism acting upstream of multiple DDR pathways. Translational research has highlighted the distinctive properties of AsiDNA™, notably its ability to abrogate tumor resistance to PARP inhibitors regardless of the genetic mutation status. AsiDNA™ has also shown a strong synergy with other tumor DNA-damaging agents such as chemotherapy and PARP inhibitors. The DRIIV-1 (DNA Repair Inhibitor-administered IntraVenously) phase I study has evaluated AsiDNA™ by systemic administration (IV) in advanced solid tumors and confirmed the active doses as well as a favorable human safety profile. The ongoing DRIIV-1b extension study is assessing the safety and efficacy of a 600 mg dose of AsiDNA™ in combination with carboplatin and then with carboplatin and paclitaxel, in patients with solid tumors who are eligible for such treatments.

**OX401** is a new drug candidate from platON™, optimized to be a next-generation PARP inhibitor acting on both the DNA Damage Response and the activation of immune response, without inducing resistance. OX401 is undergoing preclinical proof-of-concept studies, alone and in combination with immunotherapies.

Onxeo's portfolio also includes **belinostat**, an HDAC inhibitor (epigenetics). Belinostat is already conditionally FDA-approved in the US as a 2<sup>nd</sup> line treatment for patients with peripheral T cell lymphoma and marketed in the US under the name Beleodaq® (belinostat IV form).

For further information, please visit [www.onxeo.com](http://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 5.7.1.4 "Risk Factors" ("*Facteurs de Risque*") of the 2018 registration document filed with the *Autorité des marchés financiers* on April 25, 2019 under number D.19-0282, which is available on the *Autorité des marchés financiers* website ([www.amf-france.org](http://www.amf-france.org)) or on the Company's website ([www.onxeo.com](http://www.onxeo.com)).



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