

Onxeo announces final positive data from DRIIV-1 Phase 1 Study of AsiDNA™ in Advanced Solid Tumors

› Primary safety and activity endpoints met

- Favorable safety profile, maximum tolerated dose not reached, optimal active dose determined
- AsiDNA™ induced the intratumoral activation of its DNA-PK target, confirming its mechanism of action

› The full results of the study will be presented at upcoming international scientific meetings

Paris (France), May 28, 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 positive final results from the DRIIV-1 phase 1 study assessing the safety and the activity of AsiDNA™, the Company’s first-in-class DNA repair inhibitor, when administered intravenously in patients with advanced solid tumors.

Olivier de Beaumont, Medical Director of Onxeo, commented: *“DRIIV-1 successfully achieved each of its core objectives, including further demonstrating the favorable safety profile of AsiDNA™, confirming its ability to be combined with other agents and validating its mechanism of action in patients’ tumor cells through the marked activation of its targets. Importantly, the optimal active dose of AsiDNA™ has been determined and is being utilized in our ongoing DRIIV-1b study combining AsiDNA™ with chemotherapy. We intend to present the full results of the DRIIV-1 study at future scientific meetings.”*

AsiDNA™ is the first compound of a novel class of anti-tumor products. By simulating a DNA break (decoy effect), it binds to the DNA-repairing proteins, thereby preventing the recruitment of these proteins to the damaged genomic site, leading to tumor cells death.

DRIIV-1, a phase 1 dose-escalation study of AsiDNA™ administered intravenously, was designed to evaluate its toxicity profile as well as its pharmacokinetics and pharmacodynamics parameters via intratumoral activity biomarkers. The study was conducted in four centers in France and Belgium and enrolled twenty-two adult patients. All patients had metastatic cancers and were failing or progressing after one or more standard treatments with no further therapeutic options.

Five dose levels have been tested (from 200 to 1,300mg) out of the six planned. It was deemed unnecessary to test the sixth dose (1,800mg) since the therapeutic window between the optimal dose of 600mg and the highest tested dose of 1,300mg is considered sufficient.

Overall, the tolerance profile of AsiDNA™ was considered favorable by the DSMB experts, with 90% of all product-related adverse events being non-specific grade 1 and 2 events. The maximum tolerated dose (MTD) was not reached.

Most importantly, AsiDNA™ demonstrated systemic activity in DRIIV-1 through the strong activation of its targets, as evidenced by the significant increase, of two intratumoral biomarkers of DNA-PK and the decrease of a tumor proliferation biomarker. At the dose of 600mg, among the 3 patients included in the cohort, 2 patients with relapsed multi-treated metastatic colorectal cancer were controlled without progression at medical imaging at the end of the second cycle of treatment with AsiDNA™, with maintenance of treatment for 3 months.

This dose was considered optimal for the further development of AsiDNA™ in combination with chemotherapy (carboplatin and carboplatin plus paclitaxel) which started early May 2019 with the first patient treated in the phase 1b trial, DRIIV-1b.



Judith Greciet, Chief Executive Officer of Onxeo, concluded: *“The successful completion of DRIIV-1 is a major milestone for Onxeo as this study validates both the systemic activity of AsiDNA™ and its tolerance profile well-suited for combination treatments. We expect to maintain a strong development momentum and have already started the evaluation of AsiDNA™ in combination with chemotherapy in the DRIIV-1b study. Our teams are already actively working on other clinical development pathways in combination, notably with PARP inhibitors. We would like to warmly thank our investigators and their teams for their support and valuable contributions to this trial and the upcoming ones.”*

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 (proprietary, acquired or in-licensed) from translational research to clinical proof-of-concept, a value-creating inflection point appealing to potential partners.

Onxeo is developing **AsiDNA™**, a first-in-class, highly differentiated DNA Damage Response (DDR) inhibitor based on a decoy & agonist mechanism acting upstream of multiple DDR pathways. Translational research has highlighted the distinctive properties of AsiDNA™, notably its ability to oppose and even reverse tumor resistance to PARP inhibitors regardless of the genetic mutation status, and its 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 to evaluate AsiDNA™ by systemic administration (IV) in advanced solid tumors has confirmed the active doses and a favorable human safety profile. The ongoing DRIIV-1b extension study is designed to assess the safety and effectiveness of a 600 mg dose of AsiDNA™ in combination with carboplatin, and carboplatin and paclitaxel, in patients with solid tumors who are eligible for such treatments.

AsiDNA™ is the first compound generated from **platON™**, the Company’s proprietary chemistry platform of decoy oligonucleotides dedicated to generate new innovative compounds and broaden Onxeo’s product pipeline.

Onxeo’s portfolio also includes **belinostat**, an HDAC inhibitor (epigenetics). 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 under the name Beleodaq® (belinostat IV form).

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 5.7.1.4 “Risk Factors” (“Facteurs de Risque”) of the 2018 registration document filed with the *Autorité des marchés financiers* on April 5, 2019 under number D.19-0282, 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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