

Onxeo Reports Publication of Final Results of DRIIV Phase 1 Dose-Escalation Study of AsiDNA™ in Advanced Solid Tumors in the British Journal of Cancer

DRIIV was instrumental in demonstrating a good safety profile and activity via IV route and in determining the active dose used today in the ongoing DRIIV-1b and REVOCAN studies of AsiDNA™ in combination with chemotherapy and PARP inhibitors

Paris (France), August 27, 2020 – 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 particular against rare or resistant cancers, today reported the publication in the British Journal of Cancer of the final results of its DRIIV Phase 1 dose escalation study of AsiDNA™, the Company’s first-in-class DDR inhibitor, administered intravenously (IV). DRIIV was instrumental in demonstrating the good safety profile and activity of AsiDNA™ via the IV route. The active dose of 600 mg was determined to be the optimal dose for use in combination and is currently being used in the evaluation of AsiDNA™ in combination with chemotherapies (DRIIV-1b study) and a PARP inhibitor (REVOCAN study).

This open-label, dose-escalation study was conducted at 4 centers in France and Belgium (Institut Curie — Paris, Institut Claudius Régaud IUCT—Oncopôle Toulouse, Centre Léon Bérard—Lyon and Institut Jules Bordet—Brussels).

“I would like to warmly thank the teams at Onxeo and at these four renowned oncology centers who have provided the fastest access to AsiDNA™ to patients in this clinical study that represented a key milestone for Onxeo,” commented Olivier de Beaumont, Chief Medical Officer of Onxeo. “DRIIV demonstrated AsiDNA™’s favorable safety profile and validated its activity in patients’ tumor cells through a robust activation of its biological targets. Most importantly, the optimal active dose of 600 mg was determined and is currently being utilized in our ongoing combination studies. With a unique mechanism of action as decoy agonist, AsiDNA™ has the potential to both synergize with a variety of DNA-damaging agents and to abrogate resistance to targeted therapies such as PARP inhibitors. We now eagerly look forward to reporting in the coming months the topline results from DRIIV-1b, an extension study of DRIIV assessing the clinical interest of AsiDNA™ in combination with carboplatin and paclitaxel, and in early 2021, the initial data from REVOCAN, a phase 1b/2 study designed to show that the addition of AsiDNA™ to niraparib reverse the tumor resistance to this PARP inhibitor in relapsed ovarian cancer.”

The publication titled **“A Phase 1 dose-escalation study to evaluate safety, pharmacokinetics and pharmacodynamics of AsiDNA, a first-in-class DNA repair inhibitor, administered intravenously in patients with advanced solid tumours”** is available on the [British Journal of Cancer website](#).

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 γ 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 up to 1,300 mg. 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.

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.

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 chapter 3 "Risk Factors" ("*Facteurs de Risque*") of the Company's universal registration document filed with the *Autorité des marchés financiers* on April 27, 2020 under number D.20-0362, which is available on the *Autorité des marchés financiers* website (www.amf-france.org) or on the Company's website (www.onxeo.com).

Contacts

Onxeo

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

Media Relations

Nicolas Merigeau
NewCap
onxeo@newcap.eu
+33 1 44 71 94 98

Investor Relations / Strategic Communication

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