



THE ORPHAN ONCOLOGY INNOVATOR

PRESS RELEASE

Onxeo Announces Initiation of DRIIV Phase I Clinical Trial of AsiDNA™ for Treatment of Advanced Solid Tumor

- **First patient has been dosed with AsiDNA™, Company's first-in-class DNA Repair Inhibitor**
- **Study approved in France and Belgium, and conducted in three internationally-renowned clinical centers**
- **Interim results expected in second half of 2018**

Paris (France), April 24, 2018 – 06:00 pm CEST – Onxeo S.A. (Euronext Paris, NASDAQ Copenhagen: ONXEO FR0010095596), a biotechnology company specializing in the development of innovative drugs in oncology, notably against rare or resistant forms of cancer, today announced the initiation of DRIIV (*DNA Repair Inhibitor administered IntraVenously*) phase I clinical trial of AsiDNA™, its “first-in-class” DNA repair inhibitor. The aim of the study is to assess AsiDNA™ safety profile and identify its optimal clinical dose, as well as determine its active dose at the tumor level, in patients with advanced solid cancer.

First patient has been enrolled and dosed with AsiDNA™.

AsiDNA™ has a unique and innovative mechanism of action that inhibits the repair of tumor DNA damages through a decoy process by activating the enzymes involved in the signaling and repairing of tumor DNA lesions and harnessing them, thus making them unable to repair the tumor damages, which leads to the mitotic death of the cell.

The DRIIV study is being conducted at three of the most prestigious centers in France and Belgium and interim results are expected in the second half of 2018.

“The DNA-damage response approach to cancer treatment is a highly compelling approach which has been already validated with the approval of PARP inhibitors, which belongs to the same general class of DDR. AsiDNA™ has a unique mechanism of action and the potential to provide patients with an attractive therapeutic option. I look forward to further evaluating AsiDNA™ in this important clinical trial,” said Professor Christophe Le Tourneau of the *Institut Curie* in Paris, principal investigator of DRIIV.

“The initiation of DRIIV represents a significant milestone in the development of this unique asset and in enhancing the therapeutic potential of AsiDNA™,” said Judith Greciet, CEO of Onxeo. *“Importantly, while AsiDNA™ has previously shown clinical activity when injected directly into the tumor, demonstrating similar activity via systemic administration will provide the opportunity to target a vast range of cancers. We look forward to evaluating AsiDNA™ in this phase I clinical trial in order to establish the clinical benefit of this promising product candidate when administered systemically, which represents a strong value catalyst for this key asset.”*

Olivier de Beaumont, Chief Medical Officer of Onxeo, added, *“Thanks to the pre-clinical data generated to date, we have established the optimal clinical trial protocol for this phase I study and we look forward to conducting the DRIIV study with experienced oncology investigators. DRIIV is the first step in men that will enable us to effectively characterize AsiDNA™’s safety profile and determine the optimal clinical dose in order to further develop AsiDNA™, whether combined with other anticancer agents or as a monotherapy.”*



About the DRIIV study

The DRIIV clinical trial is being conducted in three of the most prestigious centers in France and Belgium: the *Institut Curie* in Paris, the *Institut Universitaire du Cancer* in Toulouse and the Jules Bordet hospital in Brussels. The study's protocol foresees a gradual increase in the administered dose with each level passed after validation by the DSMB (Data Safety Monitoring Board).

The clinical trial enrolls patients with advanced solid tumors and includes a gradual increase of the administered dose of AsiDNA™ by steps of 3 patients. For each patient, the protocol foresees an intravenous (IV) injection over 3 consecutive days followed by a weekly injection until disease progression.

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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