THE ORPHAN ONCOLOGY INNOVATOR

PRESS RELEASE

Onxeo Announces Positive Preclinical Proof of Concept Results Confirming AsiDNA™ Activity via Systemic Administration

Strong data confirms near-term plan to initiate clinical trial for AsiDNA™, Onxeo's breakthrough DNA Repair Inhibitor

Paris (France), July 05 2017 – 07h30 am 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 for the treatment of orphan diseases, in particular in oncology, today announced positive preclinical proof of concept results confirming the activity via systemic (intravenous, IV) administration of AsiDNA™, the company's first-in-class DNA repair inhibitor.

A first phase I trial (DRIMM[1]) of AsiDNA™ via local administration in melanoma previously demonstrated good tolerance and a beneficial safety profile, as well as a strong signal of efficacy. The objective of these most recent preclinical studies was to show that AsiDNA™ is also effective when administered via an IV route, which would open a wide potential of tumor types for treatment with AsiDNA™.

The generated data confirms the activity of AsiDNA™ administered intravenously, alone and in combination, as shown by the prevention of tumor growth in a murine model of triple negative breast cancer (TNBC). These data also showed a significant synergistic effect of AsiDNA™ when combined with carboplatin[2], a neoadjuvant chemotherapy used in TNBC.

AsiDNA™ via IV administration is therefore an ideal candidate for monotherapy, as well as for combination therapy with genotoxic oncology treatments, such as radio or chemotherapy, or with other DNA repair inhibitors that target a single repair pathway, such as PARP inhibitors[3].

Additionally, pharmacodynamics data generated supports AsiDNA™ unique mechanism of action whereby it acts as a decoy that attracts repair enzymes, breaks the cycle of tumor DNA repair activities and interferes with multiple repair pathways, whilst sparing healthy cells.

The activity of AsiDNA™ via systemic injection was demonstrated to be related to its ability to sequester and hyperactivate two key DNA repair proteins, DNA-PK and PARP, thus preventing their recruitment at damage sites in tumor cells.

"These positive results, combined with robust manufacturing data, provide a solid foundation from which to advance AsiDNA™ towards clinical development via a systemic route of administration," commented Françoise Bono, Chief Scientific Officer of Onxeo. "Initial data in combination with carboplatin are quite promising and we are beginning subsequent in-vivo testing of AsiDNA™ combined with PARP inhibitors."

Based on its unique attributes and the preclinical data obtained to date, AsiDNA™ has the potential to address large unmet medical needs in treating aggressive and resistant cancers, such as TNBC and ovary cancer. GlobalData estimates that the TNBC market alone will grow from €0.8 billion in 2016 to €2.1 billion by 2025.

"Achieving the in-vivo preclinical proof of concept of AsiDNA™ activity via systemic administration significantly enhances its potential compared to local administration only and represents a key milestone in the development of this promising product candidate," concluded Judith Greciet, Chief Executive Officer of Onxeo. *"Together with the optimization of the manufacturing process that the team has successfully achieved, we are on track to advance towards the clinical phase and we intend to file the phase I trial submission dossier to regulatory authorities by the end of 2017."*

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[1] Le Tourneau C et al. Br J Cancer. 2016 May 24;114(11):1199-205

[2] GeparSixto: von Minckwitz et al. Lancet Oncol 2014; CALGB 40603: Sikov et al. J Clin Oncol 2015.

[3] Jdey W et al. Clin Cancer Res. 2017 Feb 15;23(4):1001-101

About Onxeo

Onxeo is a biotechnology company developing innovative drugs for the treatment of orphan diseases in oncology, driven by high therapeutic demand in one of the fastest growing segments of the pharmaceutical industry.

Onxeo's objective is to become a major international player in the field of rare cancers. Its growth strategy is founded on the development of innovative, effective, and safe drugs based on breakthrough technologies that can make a real difference in the treatment of orphan oncology diseases and considerably improve the quality of life of patients affected by rare or resistant cancers.

Onxeo's comprehensive portfolio features a broad orphan oncology pipeline, with 3 major products in several on-going preclinical and clinical programs, alone or in combination for various cancer indications:

- **Livatag®** (Doxorubicin Transdrug™): Currently evaluated in the treatment of Hepatocellular carcinoma (HCC, also called primary liver cancer) in a phase III trial, ReLive. ReLive is a randomized, international trial designed to demonstrate the efficacy and the safety of Livatag® compared to the best available treatment chosen by the physician in 390 patients with advanced HCC after failure or intolerance to sorafenib.
- **Beleodaq®** (belinostat): FDA conditional approved in the US in 2014 under the agency's accelerated approval program as a second-line treatment for patients with peripheral T-cell lymphoma (PTCL) and currently marketed by Onxeo's partner in the US, Spectrum Pharmaceuticals; belinostat in combination with other anti-cancer agents is currently in development in first-line treatment for patients with PTCL (BelCHOP) and in solid tumors.
- **AsiDNA™**: The first-in-class siDNA (signal-interfering DNA) which has successfully undergone a proof-of-concept Phase I trial with a local administration in metastatic melanoma. The Company is currently pursuing preclinical programs to demonstrate AsiDNA activity with a systemic administration.

The Company is headquartered in Paris, France with offices in Copenhagen and in New York, and has approximately 60 employees. Onxeo is listed on Euronext in Paris, France and Nasdaq Copenhagen, Denmark (Ticker: ONXEO, ISIN Code: FR0010095596).

Learn more by visiting 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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