THE ORPHAN ONCOLOGY INNOVATOR

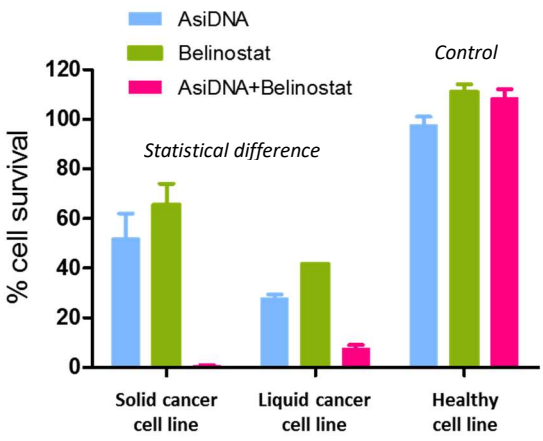
PRESS RELEASE

Onxeo announces compelling preclinical data in combination for its two innovative compounds, AsiDNA™ and belinostat.

- ***Results demonstrate a very strong synergistic effect between AsiDNA™, first-in-class DNA break repair inhibitor (DBRi), and histone deacetylase inhibitors (HDACi)***
- ***This synergy is particularly high for the combination of its two proprietary assets, AsiDNA™ and belinostat,***
- ***Potential application of AsiDNA™ in combination with any HDACi fully covered by a key worldwide patent application***

Paris (France), September 28, 2017 – 08:00 am CEST– Onxeo S.A. (Euronext Paris, NASDAQ Copenhagen: ONXEO), ("Onxeo" or the "Company"), a French biotechnology company specializing in the development of innovative drugs in oncology, in particular orphan cancers, today announced compelling results from extensive *in-vitro* preclinical studies of combinations of AsiDNA™ a first-in-class DNA break repair inhibitor (DBRi) with histone deacetylase inhibitor (HDACi), including belinostat, on various tumor cell lines. These data and potential future applications have been fully protected by a patent application filed today covering the use of AsiDNA™ in combination with any HDAC inhibitor in any treatment scheme.

Based on scientific evidence that HDAC inhibitors increase two-strand DNA breaks in tumor cells, the company has performed combination experiments demonstrating the synergistic effect of the association of AsiDNA™, its first-in-class DBRi, with HDAC inhibitors, including with its other key strategic asset belinostat, a multi-potent HDACi.



The study evaluated the efficacy of the combination on tumor cells lines and healthy cells compared to each compound alone. The results are expressed as percentage of living cells relative to non-treated condition.

The combination shows (pink bars) a very high level of efficacy (very limited detectable surviving cancer cells) compared to each compound alone, with statistical significance, on both the liquid and the solid tumor cell line. On the contrary, there was no effect on healthy cells lines, neither from compounds alone or the combination which is encouraging in terms of safety profile of the combination

Notable highlights from these *in-vitro* preclinical studies are:

- The combination is strongly synergistic and leads to tumor cells death, as shown in the above graph.
- Unlike when HDAC inhibitors are used alone, the combination of AsiDNA™ with HDACi remains synergistic over time after repeated administration: this may open the way to very relevant combination treatment schemes.
- Experiments have been repeated with other HDACi such as vorinostat, entinostat, romidepsin and generated a similar type of data showing strong synergistic effects.

The company plans to pursue this development and to begin the *in-vivo* demonstration shortly.

"These preclinical results widely open the potential of our two proprietary assets, strengthening Onxeo's position as innovation leader in DNA-targeting and epigenetics[1]*, two mechanisms of action at the forefront of oncology research and highly sought after,"* **said Judith Greciet, Chief Executive Officer of Onxeo.** *"AsiDNA™ had already shown a significant synergistic effect in combination with PARP inhibitors*[2]*, another class of DNA repair inhibitors. These new data give us the ability to leverage another type of combination of AsiDNA™ with HDAC inhibitors. As Onxeo owns both AsiDNA™ and a potent HDACi, belinostat, we enjoy the full flexibility to further advance this development in the most appropriate way for the Company."*

AsiDNA™ offers multiple paths forward, in monotherapy or combination and these recent data further expand the potential of this promising first-in-class candidate. Additional preclinical results will be disclosed on an on-going basis and the Company has planned an extensive publication plan, stating at the upcoming AACR meeting in April 2018.

Under the commercial name Beleodaq®, belinostat is already approved in the US for the 2nd line treatment of patients with peripheral T-cell lymphoma (PTCL) and marketed by Onxeo's partner Spectrum Pharmaceuticals (SPPI). For the past months, the Company has also been developing and protecting an oral formulation of belinostat. The patent application filed by Onxeo for oral belinostat would extend its protection until 2038.

These compelling preclinical data together with the long patent coverage of the oral formulation could leapfrog the current scope of belinostat to a largely wider clinical potential, far beyond PTCL, to address other types of liquid or solid tumors, with a tremendously enhanced efficacy profile when combined with AsiDNA™.

Upcoming events

October 2nd, 2017	**French Society of Financial Analysts meeting** **Portfolio Strategic Update**	**Paris, France**
October 4-5, 2017	Large & MidCap Forum	Paris, France
October 19, 2017	Portzamparc Biotech Symposium	Paris, France
October 26, 2017	Q3 results and business update	
December 13, 2017	Guggenheim Securities 5th Annual Boston Healthcare Conference	New York, USA
December 19, 2017	BioMed Invest Event	Paris, France
January 8-11, 2018	JP Morgan	San Francisco, USA
January 11-12, 2018	21st ODDO BHF Forum	Lyon, France

About Onxeo

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

Onxeo's objective is to become a major player in the field of rare or resistant cancers. Its growth strategy is to develop innovative, effective, and safe drugs based on breakthrough technologies that can make a real difference in patients' lives, by acquiring or in-licensing first-in-class or unique compounds at an early stage and bringing them through translational research and proof of concept clinical development up to value-creating inflexion points.

Onxeo's orphan oncology pipeline comprises products in several on-going preclinical and clinical programs, alone or in combination for various cancer indications.

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[1] Epigenetics refers to changes that affect gene activity and expression, without directly modifying the DNA. E.g. HDACi such as belinostat encourage DNA alterations, in particular double-strand breaks, by acting on histone and other proteins.

[2] Jdey W, et al. Clin Cancer Res. 2017 Feb 15;23(4):1001-1011

- **AsiDNA™:** a first-in-class siDNA (signal-interfering DNA) candidate which has successfully undergone a proof-of-concept Phase I trial via local administration in metastatic melanoma. Recent positive in-vivo preclinical proof-of-concept results confirmed AsiDNA™ activity via systemic administration in a murine model of triple negative breast cancer (TNBC). The Company now prepares a phase I trial of AsiDNA™ as monotherapy via systemic (intravenous) administration, expected to be submitted to the regulatory authorities by the end of 2017.
- **belinostat**: a HDAC inhibitor, conditionally FDA-approved in the US in 2014 as a 2nd line treatment for patients with peripheral T-cell lymphoma (PTCL) and marketed by Onxeo's partner in the US, Spectrum Pharmaceuticals under the commercial name of Beleodaq®; belinostat in combination with other anti-cancer agents is also in ongoing development in 1st line treatment for patients with PTCL (BelCHOP) as well as in other liquid or solid tumors. An oral formulation of belinostat should enter the phase I clinical stage early 2019. Oral belinostat would expand the asset patent protection and facilitates its use in combination with other anti-cancer agents.
- **Livatag®** is a nanoparticle formulation of the chemotherapy doxorubicin designed to facilitate the penetration of the drug into the tumor cell and increase the target DNA exposure to the drug. The ReLive phase III study in hepatocellular carcinoma demonstrated a similar effect of Livatag® as single agent, as the one showed by the best-standard-of-care group which allowed any active cancer treatment, alone or in combination. Data are under review to determine the optimal path for this asset going forward.

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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