



THE ORPHAN ONCOLOGY INNOVATOR

PRESS RELEASE

## ***Onxeo Introduces platON™, a Proprietary Chemistry Platform of Decoy Oligonucleotides***

- ***PlatON™ will generate new best-in-class DNA-targeting drug candidates starting in 2018***
- ***In parallel, the Company advances its robust development programs for AsiDNA™ and belinostat, supported by recent promising combination data and preliminary clinical data expected in 2018***
- ***Company is well-financed into early 2020, with the resources to support the current clinical development plan***

**Paris (France), October 2, 2017 – 07:00 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 introduced its proprietary chemistry platform of decoy oligonucleotides, platON™.

**PlatON™** is a proprietary chemistry platform of decoy oligonucleotides based on three components, a sequence of double strand oligonucleotides, a linker and a cellular uptake facilitator. This platform and its potential applications are fully protected by a patent family encompassing notably US patent # 9,687,557 granted in June 2017.

Each of these three components is modifiable to generate various compounds expressing different properties and/or activities, with the common feature of targeting tumor DNA repair pathways through a decoy mechanism.

**AsiDNA™**, Onxeo’s first-in-class DNA break repair inhibitor, represents the first drug candidate generated from platON™. It has already undergone a convincing phase I clinical trial via intratumoral administration and demonstrated a very promising *in-vivo* efficacy profile via systemic administration.

The Company intends to leverage platON™ to expand its pipeline with additional innovative DNA-targeting drug candidates and expects to initiate the preclinical evaluation of a new drug candidate as soon as the first half of 2018.

*“We are very excited about the potential of platON™, a truly unique platform of decoy oligonucleotides,” said **Françoise Bono, Chief Scientific Officer of Onxeo.** “As we continue to advance our two lead candidates, AsiDNA™ and oral belinostat, towards clinical development, platON™ will be generating new and unique compounds which will expand our pipeline in the highly sought-after field of DNA-targeting. We will apply our deep translational expertise, particularly in the field of oligonucleotides, to lead the preclinical and clinical development of these new compounds in the best possible way and demonstrate their potential in cancer treatment.”*

Onxeo’s strategy is to advance the development of drug candidates to proof of concept in man (phase I/phase II), the most relevant inflection points in terms of value creation for Onxeo before licensing them out.

Onxeo’s current pipeline includes two lead products, AsiDNA™, and belinostat, a potent histone deacetylase inhibitor (HDACi). Their underlying mechanisms of action, DNA-targeting and epigenetics, are among the most attractive today for pharmaceutical companies, garnering significant deal values for the past three years<sup>1</sup>.

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<sup>1</sup> Onxeo’s analysis based on Clarivate Cortellis database (only licensing deals with publicly available financial information – 01/2014-05/2017, excluding M&A) : <https://www.cortellis.com/intelligence/>



*“Our two drugs in development are based on the emerging areas of DNA-targeting and epigenetics, which are among the most promising approaches in cancer treatment research for both the pharmaceutical industry and academia today,” said Judith Greciet, Chief Executive Officer of Onxeo. “This strategic focus should enable Onxeo to create significant shareholder value in the short-term, and well into the future. In the coming quarters, we will be focused on building the most compelling data packages, including proof of concept in man, for AsiDNA™ and belinostat, alone or in combination with other anti-cancer agents, thereby creating multiple partnering opportunities. We have a sound financial visibility sufficient to cover clinical operations until early 2020, far beyond the next planned clinical milestones.”*

#### Upcoming events

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

- **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 2<sup>nd</sup> 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 1<sup>st</sup> 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 2018. 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](http://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](http://www.amf-france.org)) or on the Company's website ([www.onxeo.com](http://www.onxeo.com)).

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