

Onxeo Expands its Pipeline with New Optimized Lead OX401 Entering Proof-of-Concept Preclinical Phase

- **OX401 is the second candidate utilizing Onxeo's proprietary platform of decoy agonists, platON™**
- **OX401 is optimized to be a next-generation PARP inhibitor, designed to act on both the DNA Damage Response and the activation of immune response**
- **In vivo proof-of-concept results alone and in combination with cancer immunotherapies are expected by early Q4 2019**

Paris (France), June 20, 2019 – 6:30 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 oncology, in particular against rare or resistant cancers, today announced that its new optimized drug candidate, OX401, has started its proof-of-concept preclinical phase. Results of these studies are expected by early Q4 2019.

OX401 was designed by capitalizing on Onxeo’s expertise of oligonucleotides acting as decoy agonists and exhibits very original properties. During optimization, OX401 has demonstrated that it inhibited the DNA Damage Response by acting on PARP proteins. In parallel, OX401 activated the STING pathway, a recent and promising field of research in immuno-oncology, which makes it amenable to combinations with immuno- oncology agents such as checkpoint inhibitors.

A comprehensive patent has been filed for OX401 to protect Onxeo's intellectual property rights on this product, alone and in combination with cancer immunotherapies, until 2039.

Françoise BONO, scientific director, commented: *"This new development program represents a significant milestone for Onxeo, as it expands our R&D pipeline and prominently positions the Company at the crossroads of two of the most active fields in oncology, DNA damage response and cancer immunotherapy. Based on the experience and insights gained during the development of AsiDNA™, our first-in-class DNA repair inhibitor, we have developed and optimized OX401 to maintain its unique mechanism of action, while targeting other DNA-binding proteins and other mechanisms of tumor growth, such as the immune response. OX401 could represent a new generation of PARP inhibitors that do not have the limitations of current products, such as the induction of resistance, while providing improved biological properties, especially the activation of innate immunity within tumors."*

While the clinical relevance of PARP inhibitors is now well-established, this class still has a number of limiting factors, particularly the relatively rapid onset of resistance. Its decoy agonist mechanism of action positions OX401 as a next-generation PARP inhibitor that should not present these limitations and instead offer a lack of acquired resistance and more specificity to cancer cells.

OX401 was also developed to induce a strong immune response through the activation of the STING pathway, an area of significant interest in immuno-oncology. However, current molecules have experienced challenges, notably in terms of toxicity. OX401 is based on the same decoy agonist mechanism as AsiDNA™, Onxeo’s first-in-class DNA repair inhibitor, which showed good tolerance in the DRIIV-1 Phase 1 study, and should trigger a rapid and significant inducing effect of innate immunity against tumor cells.

Preclinical proof-of-concept results showing OX401 efficacy, alone and in combination with immunotherapy treatments, are expected early Q4 2019.



Upcoming events

- Healthtech Investor Days – Paris June 24-25, 2019 - <http://www.htid-paris.com/>

About Onxeo

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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, and its 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 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. In vivo preclinical proof-of-concept data are expected early Q4 2019.

Onxeo's portfolio also includes **belinostat**, an HDAC inhibitor (epigenetics). 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 under the name Beleodaq® (belinostat IV form).

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 5.7.1.4 "Risk Factors" ("*Facteurs de Risque*") of the 2018 registration document filed with the *Autorité des marchés financiers* on April 5, 2019 under number D.19-0282, 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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