Onxeo advances development plan for first-in-class signal interfering DNA compound AsiDNA

Near- and mid term expected milestones

Paris (France), Copenhagen (Denmark), June 27, 2016 – Onxeo S.A. (Euronext Paris, Nasdaq Copenhagen: ONXEO, "Onxeo" or "the Company"), an innovative company specializing in the development of orphan oncology therapeutics, today announced its plan for further development of AsiDNA, its first-in-class signal interfering DNA molecule which breaks the cycle of tumor DNA repair to induce cancer cell death.

In March 2016, Onxeo successfully completed the acquisition of DNA Therapeutics for its key innovative DNA repair inhibitor technology platform and lead compound AsiDNA. Following the acquisition, Onxeo has strategically assessed development options and is now accelerating a deep and comprehensive advancement plan for its newest compound both as monotherapy and in combination with anti-cancer agents.

AsiDNA: first-in-class signal interfering DNA repair compound with blockbuster potential

Onxeo's signal-interfering DNA (siDNA) product candidate, AsiDNA, is a short double-stranded DNA molecule which breaks the cycle of tumor DNA repair by interfering at the core of DNA damages, blocking multiple repair pathways, while sparing healthy cells. AsiDNA and its signal-interfering technology offer potential new treatment options for patients suffering from various types of cancer.

This technology has already demonstrated an increase in the efficacy of radiotherapy[1], radiofrequency ablation[2], and chemotherapy[3] in a variety of preclinical animal models, making it a promising candidate for both mono and combination therapy. A first-in-human Phase I/IIa trial[4] (DRIIM) performed in metastatic melanoma further demonstrated that AsiDNA molecules showed good tolerance and safety when administered intra-tumorally and subcutaneously around the tumors, with no evidence of inflammatory phenomena. Results presented at ASCO 2015[5] showed, based on 23 patients, an objective response rate (ORR) of 59% and a complete response (CR) rate of 30% compared to 10% CR with radiotherapy alone[6].

[1] Quanz et al., 2009, Berthault et al., 2011, Coquery et al., 2012, Biau et al., 2014
[2] Devun et al., 2014
[3] Devun et al. 2011, Herath et al., 2016

[4] DRIIM Phase 1/2a trial, "DNA Repair Inhibitor & Irradiation on Melanoma" NCT01469455)
[5] Abstract available at http://meetinglibrary.asco.org/content/143029-156
6 Based on literature data.

Development plan focused on systemic route administration

Preclinical: Based on these first local positive results as well as promising preclinical experiment outcomes, Onxeo is initating the development of AsiDNA through a systemic administration, enabling its potential as a therapy across a broad range of oncology indications. Preclinical programs have been initiated to further define the pharmacokinetic/pharmacodynamic profile following an intravenous administration. Results are expected in Q3/Q4 2016.

Manufacturing: In parallel, the Company is collaborating with one of the top U.S. manufacturing expert facilities for complex life science products, in order to optimize the manufacturing process of AsiDNA. Overall, the goal is to improve costs and production duration for future large-scale clinical development and industrialization. First results for this process development are expected in Q4 2016.

Clinical: Based on the already deep knowledge generated and the study outcomes set forth above, Onxeo is exploring opportunities to accelerate the clinical development of AsiDNA and is planning to launch the Company's first clinical trial of AsiDNA as soon as 2017 to assess safety and first indication of anti-cancer activity of AsiDNA as monotherapy via systemic administration.

Judith Greciet, CEO of Onxeo, commented, *"We have built an ambitious plan for AsiDNA to demonstrate its value in several types of cancer through a systemic administration. This plan aims at accelerating development with presentation of preclinical data within the next 6 months and a view to entering the clinic shortly therafter. First clinical data of AsiDNA in systemic use represent a major step in our development strategy as these results have the ability to confirm the tremendous potential of the product. Signal interfering DNA repair technology is increasingly becoming one of the most innovative research fields and such data, as soon as 2017, could support sound value creation for AsiDNA, for patients and for Onxeo as a company."*

Expected AsiDNA newsflow

Q3/Q4 2016: Results of preclinical trial of AsiDNA
Q4 2016: Optimization of manufacturing process
2017: Launch of AsiDNA clinical trial in monotherapy

About Onxeo

Onxeo is a leading developer of orphan oncology drugs. The Company is focused on developing innovative therapeutics for rare cancers, one of the fastest growing markets in the healthcare industry with high, unmet medical needs. Onxeo's vision is to become a global leader and pioneer in oncology, with a focus on orphan or rare cancers, by developing advanced, effective, and safe therapeutics designed to improve the lives of patients. Onxeo's comprehensive portfolio features a broad orphan oncology pipeline, with four independent programs in various stages of clinical development, including Onxeo's first approved orphan oncology drug, Beleodaq®. The Company is headquartered in Paris, France and has approximately 50 employees. Onxeo is listed on Euronext in Paris, France (Ticker: ONXEO, ISIN Code: FR0010095596) and Nasdaq Copenhagen, Denmark (Ticker: ONXEO).

Onxeo's orphan oncology products are:

- **Livatag®** (Doxorubicin Transdrug™): Currently being evaluated in a Phase III trial (ReLive) in patients with hepatocellular carcinoma (primary liver cancer); and in combination with other cancer agents in first-line HCC
- **Beleodaq®** (belinostat): FDA-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 cancer agents is currently in development in first-line treatment for patients with PTCL (BelCHOP) and in other solid tumors
- **AsiDNA**: The first-in-class siDNA (signal-interfering DNA) which has successfully undergone a proof-of-concept Phase I/IIa trial in metastatic melanoma

- **Validive®** (Clonidine Lauriad®): Positive final results from a Phase II trial in head and neck cancer patients with severe oral mucositis
  In addition, Onxeo has successfully developed and registered two non-cancer products which are currently being commercialized in the U.S. and Europe.

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