



THE ORPHAN ONCOLOGY INNOVATOR

PRESS RELEASE

## ***Full-year 2016 Results and Outlook for 2017***

### ***Reinforcement of Onxeo's strategic positioning in the development of innovative oncology treatments***

- **Acquisition of DNA Therapeutics and the AsiDNA™ product, a first-in-class candidate in the new area of preventing tumor cells from repairing their DNA**
- **Enrolment for the phase III Livatag® study in advanced primary liver cancer completed on schedule to deliver preliminary results mid-2017**
- **Cash and cash equivalents of €29.2 million at December 31, 2016, providing financial visibility through to 2018.**

**Paris, March 7, 2017 – 6:30 pm CET.** Onxeo S.A. (Euronext Paris, NASDAQ Copenhagen: ONXEO), a clinical-stage biotechnology company specializing in the development of innovative drugs for the treatment of orphan diseases, in particular in oncology, today released its consolidated results for 2016 and its outlook for 2017.

*"2016 was a landmark year for Onxeo in several ways. From a clinical standpoint, we continued enrolment for our ReLive trial in the treatment of primary liver cancer with Livatag®, our first drug candidate. This has now been completed on schedule to meet our target of announcing the results of this major study during summer 2017. With Beleodaq® already approved for use in the United States as a second line therapy for peripheral T-Cell lymphoma (PTCL), we continued our efforts to extend its use to other indications and commenced work on developing an oral formulation. From a strategic standpoint, the highlight of the year was the acquisition of the DBait technology and its lead compound AsiDNA™ that establishes Onxeo as a pioneer in the high-potential therapeutic area of inhibiting the repair of tumor cells' DNA. To support the ramp-up in our strategic programs, we have enhanced the performance of our preclinical and clinical operations. All these developments were achieved without compromising on our rigorous cost control, which has ensured that we have the required financial resources to support our future growth,"* commented Judith Greciet, Chief Executive Officer of Onxeo.

### **2016 FINANCIAL HIGHLIGHTS**

Recurring revenues grew by 26% compared with 2015. They totaled €3.5 million thanks to the commercialization of Beleodaq® in the United States by our partner Spectrum Pharmaceuticals.

Operating expenses came to €27.6 million, with 57% accounted for by R&D, as ReLive, the international clinical trial of Livatag®, progressed on schedule and AsiDNA™ entered the pipeline. General and administrative expenses remained under control thanks to Onxeo's strict cost control policy.

The research tax credit for 2016, payment for which will be requested in 2017, amounted to €4 million in France and Denmark and was deducted from operating costs in accordance with IFRS.

Onxeo held €29.2 million in consolidated cash at December 31, 2016, demonstrating its healthy financial position that provides visibility until the beginning of 2018. This cash balance includes €12.5 million in net proceeds from the capital increase resulting from the late September 2016 private placement.



Cash consumption amounted to €20.5 million of cash in line with program developments during the year.

Onxeo's financial statements for the 2016 financial year have been reviewed by its Statutory Auditors and were approved by the Board of Directors on March 7, 2017.

## CONSOLIDATED FINANCIAL STATEMENTS FOR FY 2016

Consolidated financial statements (IFRS)	FY to Dec. 31, 2016	FY to Dec. 31, 2015
<i>In thousands of euros</i>		
<b>Revenues, o/w</b>	4,423	3,481
<i>Recurring revenues</i>	3,454	2,733
<i>Non-recurring revenues</i>	969	749
<b>Operating expenses, o/w</b>	(27,591)	(25,657)
<b>Research and development expenses</b>	(18,075)	(16,350)
<i>Research tax credit</i>	3,955	3,718
<i>General &amp; administrative expenses</i>	(13,471)	(13,025)
<b>Operating income/(loss) before non-recurring items</b>	(23,168)	(22,365)
<b>Non-recurring operating income/(loss)</b>	(43)	(189)
<b>Net financial income/(expense)</b>	1,106	602
<b>Income tax expense</b>	(566)	2,353
<b>Net income</b>	(22,671)	(19,409)

## 2016 HIGHLIGHTS

### ▪ DRUG CANDIDATE PIPELINE IN ONCOLOGY

#### ○ [Livatag®](#)

During 2016, Onxeo accelerated enrollment in the phase III ReLive trial evaluating Livatag®, the most advanced drug in its pipeline, in the second-line treatment of advanced hepatocellular carcinoma (HCC). The Company presented the results of a study of Livatag®'s mechanism of action at the AACR Annual Meeting 2016. These results show preferential affinity for the liver, confirming the drug's potential in advanced hepatocellular carcinoma.

During the financial year, Onxeo actively continued its development program for this asset in other indications and/or in combination with other agents. It announced encouraging pre-clinical results for Livatag® in combination with new immuno-oncology agents of various classes, such as PD-1 and CTLA-4 checkpoint inhibitors, and as a monotherapy for pancreatic cancer.

Onxeo commenced collaboration in 2016 with the University of Navarra's CIMA (*Centro de Investigación Médica Aplicada*) immunology research program and hepatology department in Spain, in addition to other partnerships already established in Europe.

#### ○ [AsiDNA™](#)

On March 25, 2016, Onxeo announced that it had completed the acquisition of DNA Therapeutics and thereby secured ownership of an innovative technology acting on the DNA repair mechanisms of tumor cells (DBait).

This acquisition has bolstered its portfolio of orphan drugs for use in oncology and firmly established Onxeo in DNA repair, a ground-breaking new avenue of scientific research in oncology. The technology developed by DNA Therapeutics acts at the early stage of the multiple DNA repair pathways, by detecting and signaling damage, thereby interrupting the repair cycle of tumoral DNA, without affecting healthy cells.



AsiDNA™, the first-in-class product from this new technology, has already demonstrated an acceptable tolerance profile and safety with intra-tumoral and sub-cutaneous administration as a combination therapy with radiotherapy in a phase I/IIa trials in patients with metastatic melanoma.

In July 2016, Onxeo was notified by the US Patents and Trademarks Office that it had been allowed a key patent for AsiDNA™, extending its protection in the United States until 2031.

The Group announced an ambitious development plan for this new asset in the second quarter of 2016.

- [Beleodaq®](#)

Onxeo continued its partnership with Spectrum Pharmaceuticals (Nasdaq : SPPI), who holds the US rights for development and commercialization, to finalize the clinical development in peripheral T-Cell lymphoma (PTCL) treatment, especially the completion of the Phase III design for first line treatment. The development is pursued by Spectrum, who holds the US AMM. Discussions are still ongoing between the two companies as well as with the agencies in order to confirm the start of this study.

In line with its clinical strategy, Onxeo intends to evaluate other potential tumor-related indications for Beleodaq® in combination with immuno-oncology agents (checkpoint inhibitors) in addition to PTCL, for which Beleodaq is already approved in the United States. The first pre-clinical studies have shown that growth is halted completely in tumors treated with Beleodaq in combination with these immuno-oncology agents. The Company thus continues to pursue this promising program.

Furthermore, Onxeo announced the development of an oral formulation of belinostat, currently available solely for intravenous administration. This new formulation would significantly benefit patients and doctors alike, facilitating administration, promoting observance and not requiring any assistance from medical personnel. It would also enable Onxeo to extend belinostat's patent protection and enhance the appeal of its development as a combination therapy with other drugs in additional indications.

Lastly, Onxeo has continued to expand Beleodaq®'s commercial expansion in the PTCL indication by entering into an exclusive licensing agreement with Pint Pharma for the commercialization of Beleodaq® in PTCL in several key South American countries. In addition to an initial upfront payment, the agreement provides for regulatory and commercial milestones, and double-digit royalties on net sales of Beleodaq® in these territories, adding up to over \$20 million.

- [Other products](#)

Onxeo confirmed in the first quarter of 2016 its strategic decision to proceed with the development of Validive® only with the support of an industrial partner.

- **ORGANIZATION AND GOVERNANCE**

In March 2016, the Group announced that it had established Onxeo US Inc., a US subsidiary based in New York and run by Philippe Maître, Executive Vice President & Chief of US Operations.

Joseph Zakrzewski was confirmed as Chairman of the Board of Directors in April 2016. He possesses over 25 years of pharmaceutical industry experience, mostly in the United States. Dr. Jean-Pierre Kinet from Harvard and Dr. Jean-Pierre Bizzari, both internationally renowned experts in oncology, joined Onxeo's Board of Directors as independent directors. These appointments were confirmed by shareholders at the Annual General Meeting on April 6, 2016.

## POST CLOSING EVENTS

January 24, 2017 Finalization of the enrollment of the planned 390 patients in the ReLive study to evaluate Livatag® as second-line treatment for hepatocellular carcinoma. The availability of the preliminary results in mid-2017 is confirmed.



- January 31, 2017 Partnership with the Institut Curie to study the benefits of combining radiotherapy, AsiDNATM, Onxeo's tumoral DNA repair inhibitor, and immunotherapy in the treatment of cancer.
- February 13, 2017 USPTO Notice of Allowance for a new patent covering broader claims related to AsiDNA™ and comparable molecules.
- February 28, 2017 Onxeo appoints seasoned executives to accelerate preclinical and clinical development: Françoise Bono, PhD, named Chief Scientific Officer to lead preclinical activities and Olivier de Beaumont, MD, MBA, appointed Chief Medical Officer to lead clinical development and operations, medical and regulatory affairs.

## OUTLOOK FOR 2017

### ■ DEVELOPMENT STRATEGY

In 2017, Onxeo will pursue its growth strategy leveraging on its most innovating and promising programs. The main growth catalysts are expected to be:

- Livatag® (Doxorubicine Transdrug®): preliminary efficacy results from phase III of the ReLive trials due mid-way through the year, results of preclinical studies of Livatag® evaluating the drug's potential in new indications.
- Beleodaq® (belinostat): preparations with US partner Spectrum Pharmaceuticals for the extension of the indication to first-line treatment of PTCL, development of a new orally administered formulation by the Company and assessment of the benefits of combining Beleodaq with other anti-cancer agents, including immuno-oncological agents for the treatment of non-PTCL tumors.
- AsiDNA™: *in vitro* proof of concept for the systemic route (intravenous) administration, evaluation of the benefits of combining AsiDNA™ with other anti-cancer agents in various animal tumor models, finalization of the optimization of AsiDNA™ manufacturing processes prior to implementation of a phase I clinical trial to demonstrate AsiDNA™ activity by systemic route administration.

The Company intends to pursue its external development based on identification and acquisition of early stage, highly innovative therapeutic candidates in rare and resistant cancers, with the aim of taking them forward to the development milestone that will generate the most value for the company and its shareholders.

**UPCOMING EVENTS – S1 2017**

March 8	French Society of Financial Analysts meeting	Paris, France
March 21-22	Portzamparc Midcaps Forum	Paris, France
April 1-5	AACR Annual meeting	Washington, DC, USA
April 7	BioCentury – Future Leaders in the Biotech Industry	New York, NJ, USA
April 26	Shareholders' Annual General Meeting	Paris, France
April 27	Q1 results	-
May 11-12	Meet2Win 2017 - Oncology	Bordeaux, France
May 25	BTIG Biotech Executive Forum	Boston, MA, USA
June 8	Kepler Biotech Days	Paris, France

**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.

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

Learn more by visiting [www.onxeo.com](http://www.onxeo.com).

**Disclaimer**

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 Risk Factors ("Facteurs de Risque") section of the 2015 Reference Document filed with the AMF on April 29, 2016, which is available on the AMF website (<http://www.amf-france.org>) or on the company's website ([www.onxeo.com](http://www.onxeo.com)).

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