Advancing innovation towards breakthrough cancer therapies

LISTED
EURONEXT | Paris
NASDAQ | Copenhagen

EPA: ONXEO

November 2017
Important Information

IMPORTANT: You must read the following before continuing. In accessing this document, you agree to be bound by the following terms and conditions.

This document has been prepared by Onexxo SA (together with its subsidiaries, the “Group”) and is for information purposes only. The content of this document is provisional and for information purposes only and is not to be construed as providing investment advice. The information, statements and opinions contained in this document (the “Information”) are provided as of the date of this document only and may be subject to significant changes at any time without notice. Neither the Group, nor its advisors, nor any other person is under any obligation to update the Information. Subject to applicable law, none of the Company or its advisors accepts any responsibility whatsoever and makes no representation or warranty, express or implied, as to the fairness, accuracy, completeness or correctness of the Information. The Information has not been subject to independent verification and is qualified in its entirety by the business, financial and other information that the Group is required to publish in accordance with the rules, regulations and practices applicable to companies listed on Euronext Paris, including in particular the risk factors in the Company’s Registration Document filed with the French Financial Markets Authority (Autorité des marchés financiers) under number D.17-0423 on April 24, 2017, in any other periodic report and in any other press release, which are available free of charge on the websites of the Group (www.onexxo.com) and/or the AMF (www.amf-france.org).

This document contains information on the use of the Group’s products and its competitive position. Some of the Information is from third parties. While this third party information has been obtained from sources believed to be reliable, there is no guarantee of the accuracy or completeness of such data. In addition, certain of the industry and market data comes from the Group’s own internal research and estimates based on the knowledge and experience of the Group’s management. While the Group believes that such research and estimates are reasonable and reliable, they, and their underlying methodology and assumptions, have not been verified by any independent source for accuracy or completeness and are subject to change without notice. Accordingly, undue reliance should not be placed on any of the industry, market or competitive position data contained in the Information.

The Information is not directed to, or intended for distribution to or use by, any person or entity that is a citizen or resident of, or located in, any locality, state, country or other jurisdiction where such distribution or use would be contrary to law or regulation or which would require any registration or licensing within such jurisdiction. The Information does not constitute or form part of, and should not be construed as an offer or the solicitation of an offer to subscribe for or purchase of any securities. No public offering of securities may be conducted in France prior to the delivery by the French Financial Markets Authority of a visa on a prospectus that complies with the provisions of Directive 2003/71/EC as amended. This document is for information purposes only and does not constitute an offering document or an offer of securities to the public in the United Kingdom to which section 85 of the Financial Services and Markets Act 2000 of the United Kingdom applies. Securities may not be offered or sold in the United States absent registration under the US Securities Act of 1933, as amended, or an exemption from registration thereunder.

This document contains certain forward-looking statements. All statements in this document other than statements of historical fact are or may be deemed to be forward looking statements. These statements are not guarantees of the Group’s future performance. These forward-looking statements relate without limitation to the Group’s future prospects, developments, marketing strategy regulatory calendar, clinical milestones, assumptions and hypothesis, clinical development approach and financial requirements and are based on analyses of earnings forecasts and estimates of amounts not yet determinable and other financial and non-financial information. Forward-looking statements are subject to a variety of risks and uncertainties as they relate to future events and are dependent on circumstances that may or may not materialize in the future. Forward-looking statements cannot, under any circumstance, be construed as a guarantee of the Group’s future performance as to strategic, regulatory, financial or other matters, and the Group’s actual performance, including its financial position, results and cash flow, as well as the trends in the sector in which the Group operates, may differ materially from those proposed or reflected in the forward-looking statements contained in this document. Even if the Group’s performance, including its financial position, results, cash-flows and developments in the sector in which the Group operates were to conform to the forward-looking statements contained in this document, such results or developments cannot be construed as a reliable indication of the Group’s future results or developments. The Group expressly declines any obligation to update or to confirm projections or estimates made by analysts or to make public any correction to any prospective information in order to reflect an event or circumstance that may occur after the date of this document.
INVESTMENT CASE
From cutting-edge science to breakthrough products

DIFFERENTIATED SCIENCE AND BUSINESS MODEL targeting unique mechanisms of action in DNA-targeting with strategy to partner post Proof-of-Concept

PROPRIETARY platON™ CHEMISTRY PLATFORM will be leveraged to generate new breakthrough oncology compounds.

ROBUST DEVELOPMENT PIPELINE with two product candidates entering clinic in 2018 targeting multiple potential indications

MULTIPLE NEAR-TERM CATALYSTS with data from Phase I trials expected within the next 12-18 months

Developing breakthrough DNA-targeting therapies for unmet cancer treatment needs
Differentiated business model

Develop products to proof-of-concept/optimal inflection point, compelling for pharma partners, based on a strong translational expertise, to optimize value and time to assets monetization

- Build a diversified pipeline with first-in-class programs
  - Innovative programs in DNA Repair and Epigenetics, two of the most sought-after areas in oncology
  - platON™, a unique chemistry platform generating new DNA-targeting compounds

- Leverage deep translational expertise to minimize time and risks from preclinical to clinical

- Proven track record in executing value-creating business development-related transactions
A complementary and experienced management team

Nicolas FELLMAN
CFO
- Formerly Pfizer, Ernst & Young

Nicolas FELLMAN
CFO
- Formerly Pfizer, Ernst & Young

Philippe MAITRE
EVP Onxeo Inc., CBDO
- Leads the US subsidiary
- Formerly Aventis, PPD, mAbRx

Philippe MAITRE
EVP Onxeo Inc., CBDO
- Leads the US subsidiary
- Formerly Aventis, PPD, mAbRx

Judith GRECIET (Pharm.D)
CEO since 2011
- Formerly Wyeth, Eisai
- Eisai France President until 2011

Judith GRECIET (Pharm.D)
CEO since 2011
- Formerly Wyeth, Eisai
- Eisai France President until 2011

Françoise BONO (PhD)
CSO
- Sanofi, Evotec
- Evotec’s EVP until 2016

Françoise BONO (PhD)
CSO
- Sanofi, Evotec
- Evotec’s EVP until 2016

Olivier DE BEAUMONT (MD)
CMO
- Formerly Senior VP
Stallergenes Greer, Quintiles, Aventis

Olivier DE BEAUMONT (MD)
CMO
- Formerly Senior VP
Stallergenes Greer, Quintiles, Aventis

Demonstrated track record in product development and business development
**DNA Repair and Epigenetics**

2 of the most sought-after mechanisms of action in oncology

---

**Number of deals in Oncology**

<table>
<thead>
<tr>
<th></th>
<th>24</th>
<th>24</th>
<th>17</th>
</tr>
</thead>
<tbody>
<tr>
<td>Deals with published financials/ upfront</td>
<td>7/5</td>
<td>14/12</td>
<td>8/8</td>
</tr>
</tbody>
</table>

**Phases I and II drive the highest deals value**

---

**DNA Repair and Epigenetics**

*Onxeo's analysis based on Clarivate Cortellis (only licensing deals with publicly available financial information, excluding M&A - 2013-06/2017): [https://www.cortellis.com/intelligence/](https://www.cortellis.com/intelligence/)*
### Programs

<table>
<thead>
<tr>
<th>Programs</th>
<th>INDICATION</th>
<th>PRECLINICAL</th>
<th>PHASE I</th>
<th>PHASE II</th>
<th>PHASE III</th>
<th>UPCOMING MILESTONES</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>platON™</strong></td>
<td>GENERATION OF NEW DNA-TARGETING COMPOUNDS</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Next compound H1 2018</td>
</tr>
<tr>
<td>Proprietary chemistry platform of decoy oligonucleotides</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td><strong>AsiDNA™ IV</strong></td>
<td>Solid tumors</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Phase I filing Q4 17</td>
</tr>
<tr>
<td><strong>AsiDNA™ + PARPi</strong></td>
<td>Solid tumors</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Ready to deliver Proof-of-Concept (PoC) in man end 2018</td>
</tr>
<tr>
<td><strong>AsiDNA™ + chemo/radio</strong></td>
<td>Solid tumors</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>PoC confirmed via IT (DRIIM study)</td>
</tr>
<tr>
<td><strong>AsiDNA™ + belinostat /HDACi</strong></td>
<td>Solid tumors</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Ready for clinical phase I 2018</td>
</tr>
<tr>
<td><strong>Oral belinostat</strong></td>
<td>Liquid &amp; solid tumors</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Phase III required by the FDA from SPPI as MA holder in 2nd line</td>
</tr>
<tr>
<td><strong>Beleodaq® + CHOP</strong></td>
<td>PTCL¹ 1st line</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td>Looking for a partner to explore other options (HBV, 1st line, combo...)</td>
</tr>
<tr>
<td><strong>Livatag®</strong></td>
<td>Hepatocellular carcinoma</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

**Notes:**

1. IT: intratumoral – IV: intravenous
2. Beleodaq®: commercial brand name of belinostat (IV form) in the US in PTCL
3. CHOP: Cyclophosphamide, Vincristine, Doxorubicine, Prednisone
4. PTCL: Peripheral T-cell lymphoma – a rare form of blood cancer
5. SPPI: Spectrum Pharmaceuticals, Onxeo's partner and Market Authorization holder in the US for the use of Beleodaq in the treatment of PTCL in 2nd line
Leverage proprietary decoy oligonucleotides technology to generate new breakthrough compounds in oncology.
platON™: Proprietary Chemistry Platform of Decoy Oligonucleotides

All three components of the platform can be acted upon to obtain new compounds with different properties and/or activities

A powerful and versatile platform to generate breakthrough compounds acting on intracellular DNA binding targets

* Trademark pending
platON™: expanding Onxeo’s ability to generate new candidates

- AsiDNA™, first drug candidate from platON™ to enter Phase 1 in Q1 2018
- Next compounds planned from H1 2018

Combine deep biology insight with leading translational expertise and capabilities in oncology to deliver new, best-in-class drugs
AsiDNA™

A first-in-class compound targeting tumor DNA break repair pathways

First compound issued from platON™ decoy oligo-nucleotides platform (acquired with DNA Therapeutics in 2016)

Clinical proof-of-concept achieved (DRIIM phase 1 study - intratumoral)

Preclinical proof-of-concept by systemic administration (IV) achieved
Clinical translation ongoing (PK/PD relationship)

Large potential in multiple indications, alone (stratification biomarkers under validation) & in combination (eg. radio/chemotherapy/ PARPi)

NEXT MILESTONE
AsiDNA™ IV
Phase 1 filing Q4 2017
Prevention of tumor DNA repair is a leading-edge field of research in oncology

- Potential new cancer treatment paradigm: Nobel Prize 2015 on DNA repair mechanisms

- Many cancer treatments or genetic instabilities induce DNA damaging but tumor cells survive by repairing DNA damage, leading to resistance

- DNA break repair inhibitors treatments lead to cancer cell death alone (on genetically instable tumors) or in combination with DNA damaging agents
AsiDNA™: A first-in-class compound in DNA Break Repair inhibition

32 bp DNA duplex with a 5´-Chol-TEG & a non-nucleotidic loop

- Double-stranded 32 bp DNA is tethered with a loop to prevent disassociation\(^1\)
- Phosphorothioate substitutions at the 5´ and 3´ ends to prevent degradation\(^1\)
- Efficient nuclear uptake of the DNA is mediated via a covalently linked cholesterol molecule\(^2\)

**Simple, elegant, unique and safe**

AsiDNA™ - A mechanism of action unlike any other: DNA break repair inhibition through an agonist mechanism

Recruitment of DNA damage signaling and repair proteins to the sites of genomic damage is one of the early events in tumors DNA repair.

AsiDNA™ is a double stranded DNA molecule (decoy oligo-nucleotide) that mimics double stranded DNA breaks to interfere with DNA repair, redirecting repair enzymes away from sites of tumor DNA damage.

Tumoral cells (deficient cell cycle control) keep dividing with damaged DNA leading to DNA fragmentation and loss and then mitotic death.

Healthy cells (proficient cell cycle control) stop dividing and reducing transcription, waiting for the disappearance of false signal to resume dividing.

Healthy cells and tissues are insensitive to AsiDNA™

Healthy cells insensitivity

- AsiDNA™ triggers the activation of DNA PK and the inhibition of enzyme recruitment in normal cells as well in cancer cells, while not inducing the death of normal cells\(^1\)

Healthy tissues insensitivity in phase I trial (DRIIM study)

- AsiDNA™ shows no toxicity on healthy tissues even after irradiation\(^2\)

---

AsiDNA™: First clinical outcomes from DRIIM Phase I study 
(*intratumoral administration + radiotherapy for metastatic melanoma*)

- **Proof of concept established**
  - Overall response rate = 59%
  - Complete response = 30%
    (CR from low-dose radiotherapy alone less than 10%)%)
  - Partial response = 29%
  - Durable response (up to 12 months follow up period)

- **Well tolerated compound**
  - Absence of immune response

- **PK/PD properties**
  - Tumor responses statistically correlated to plasmatic exposure of AsiDNA

---


---

**Responses / Exposure (AUC)**

<table>
<thead>
<tr>
<th>Dose level</th>
<th>&lt; AUC &gt;</th>
</tr>
</thead>
<tbody>
<tr>
<td>16 mg</td>
<td>1.0 µg/mL*h</td>
</tr>
<tr>
<td>32 mg</td>
<td>2.0 µg/mL*h</td>
</tr>
<tr>
<td>64 mg</td>
<td>3.7 µg/mL*h</td>
</tr>
<tr>
<td>96 mg</td>
<td>~5.7 µg/mL*h</td>
</tr>
<tr>
<td>48 mg</td>
<td>7.2 µg/mL*h</td>
</tr>
</tbody>
</table>

---

**PK/PD properties**

- Tumor responses statistically correlated to plasmatic exposure of AsiDNA

---

**Proof of concept established**

- Overall response rate = 59%
- Complete response = 30%
  (CR from low-dose radiotherapy alone less than 10%)%)
- Partial response = 29%
- Durable response (up to 12 months follow up period)
AsiDNA™: *In vivo* proof of therapeutic efficacy
(*intravenous* administration in Triple Negative Breast Cancer murine model)

- AsiDNA™ **sequesters** and sustainably **hyperactivates** the key DNA repair proteins DNA-PK thus preventing effective DNA repair in tumors.

- Demonstrated therapeutic efficacy of AsiDNA™ alone administered intravenously.

*As measured by γH2AX phosphorylation*
AsiDNA™: In vivo efficacy on both BRCA mutated and non-mutated tumors vs. PARP inhibitors
*(intratumoral administration in Triple Negative Breast Cancer murine model)*

- **Effect of AsiDNA and olaparib (PARPi) on BRCA -/- (mutated) Triple Negative Breast Cancer**
- **In contrast to PARPi, AsiDNA displays strong efficacy in BRCA+/+ (non-mutated) tumors**¹

**Synergy on mutated BRCA, and no limitation in non-mutated tumors**

AsiDNA™ Development Strategy

- **Confirm AsiDNA™ activity via systemic administration (IV)**
  - Biomarkers activity and stratification (micronuclei, γH2AX)
  - Ongoing in vivo preclinical in several tumors (combo with PARPi, HDACi...)
  - **H1 2018**

- **First-in human Phase I AsiDNA™ IV in advanced malignancies**
  - Confirm proof of mechanism in human, determine optimal clinical dose
  - Translational clinical outcomes: relationship between exposure, activity & safety
  - **CTA Filing Q4 2017**

- **Clinical development program extension in solid/liquid tumors**
  - AsiDNA™ IV in combination with DNA damaging agents (PARPi, HDACi...)
  - AsiDNA™ IV alone for any genetically unstable tumors
  - **End 2018/Early 2019**

Proprietary technology (Method of Use) patent until 2024
Drug product and related compounds protected until 2031

November 2017
AsiDNA™: Our objectives

- Propose a new paradigm for cancer treatment
  - Open a new avenue for cancer treatment with a disruptive product / mechanism of action

- Confirm strong and promising activity
  - Capitalize on a set of preclinical results showing promising activity via systemic administration, in monotherapy or in combination with other anti-cancer therapies

- Deliver preliminary results in man, expected from H2 2018
  - Pursue a clear and efficient development plan to deliver early and compelling results

- platON™
  - Leverage our expertise with AsiDNA™ to create new compounds from platON™ platform

A first-in-class drug candidate with a very differentiated mechanism of action and high value potential
belinostat (Beleodaq®)  
HDAC inhibitor

Acquired through the merger with Topotarget (2014)

Fast Track / Orphan Drug Designation (US/EU)

FDA conditional approval in 2nd line PTCL (2014) for Beleodaq® IV

Partnered to Spectrum (SPPI) for the US, & to Pint Pharma for South America

Ongoing development of an oral formulation
Ongoing exploration of combos in liquid & solid tumors

NEXT MILESTONE
Phase 1 (oral / mono) filing Q4 2017
Beleodaq® (belinostat IV) already approved and marketed in Peripheral T-Cell Lymphoma (PTCL)

Beleodaq® in PTCL

Peripheral T-cell lymphoma
- A subtype of non-Hodgkin’s lymphoma (NHL) which affects T-cells with 17,000 to 27,000 incident cases\(^1\) in key pharmaceutical markets

Regulatory status
- Fast Track / Orphan Drug Designation (US & EU); FDA conditional approval in 2\(^{nd}\) line PTCL in 2014 following successful Phase II Belief Study
- Commercialized in the US by Spectrum Pharma (SPPI) since July 2014

Clinical development

Successful Phase II Belief Study (n = 129)\(^2\)
- Pivotal for FDA conditional approval
- Favorable safety profile

Successful Phase I study of Beleodaq® + CHOP* (n=23) in 1\(^{st}\) line PTCL

Phase III clinical study of Beleodaq® + CHOP in PTCL 1\(^{st}\) line
- FDA requirement post conditional approval in 2\(^{nd}\) line
- Under preparation by SPPI** (as market authorization holder in the US)

Beleodaq® is the brand name of belinostat via intravenous (IV) administration in PTCL in the US

\(^1\) International Peripheral T-Cell and Natural Killer/T-Cell Lymphoma Study, (J Clin Oncol 26 :4124-4130) and GLOBOCAN 2012, IARC data.

* CHOP: Cyclophosphamide, Vincristine, Doxorubicine, Prednisone - ** Spectrum Pharmaceuticals

November 2017
Rationale for combining HDACi\(^1\) + DBRi\(^2\)

- HDACi induce histone hyperacetylation and ROS* generation that cause structural alterations in chromatin which may expose portions of DNA that are normally protected by heterochromatin from DNA-damages.

Rationale for belinostat

- Recognized efficacy and superior safety profile vs. other products approved in R/R PTCL makes it more amenable to drug combination.

---

1. HDACi: histone deacetylase inhibitors
2. DBRi: DNA Break Repair inhibitors
3. ROS: radical oxygen species

Adapted from J.H. Lee et al, PNAS – vol 107, 33, 2010
Belinostat: strong increase of DNA breaks on tumor cells, no effect on healthy cells

\( \gamma H2AX \) quantification (biomarker)
**Belinostat: strong synergy in combination with DBRi**
*(preclinical in vitro studies on tumor cell lines)*

- Synergistic effect of belinostat combined with various DNA Break Repair inhibitors (DBRi):

  - **Belinostat: strong synergy in combination with DBRi**
    - Synergistic effect of belinostat combined with various DNA Break Repair inhibitors (DBRi):
      - **+ CheK1 inhibitor**
        - (LY2603618)
      - **+ AsiDNA™**
      - **+ ATR inhibitor**
        - (AZD6738)

*Almost no detectable surviving tumor cells*

**Increased cell lethality in different tumor cell lines (solid and liquid)**
*No lethality observed in healthy cells with belinostat*
**Oral belinostat formulation development**

- New formulation technology (Amorphous spray dried dispersion)
  - Improved bioavailability by x4
  - Extension of indications (combos, maintenance) & patients’ access (outpatients)

**Significant potential competitive advantages over IV formulations**

*Belinostat oral formulation extends IP protection until 2038*
belinostat: our objectives

Execute rigorous development plan to expand potential beyond PTCL
- Complete the successful ongoing development plan of a compound already approved in an orphan indication

Leverage the benefits in combination therapies
- A combination protocol opens a wide range of potential cancer indications
- An already approved IV form could speed up proof of mechanism of a combination

Harvest the value of solid patent protection
- Patent filed to extend protection until 2038 with oral formulation

Significant competitive advantages in this class of product and high value potential in combinations

November 2017
Financial figures and next milestones
### Financials

**Shares**

<table>
<thead>
<tr>
<th>Description</th>
<th>Quantity</th>
</tr>
</thead>
<tbody>
<tr>
<td>Shares outstanding (10/31/17)</td>
<td>50.7M</td>
</tr>
<tr>
<td>Average daily stock turnover (9 months 2017)</td>
<td>470,092 shares</td>
</tr>
<tr>
<td>Market cap</td>
<td>+/-70M€</td>
</tr>
</tbody>
</table>

**CASH POSITION (09/30/17)**

<table>
<thead>
<tr>
<th>Description</th>
<th>Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>Cash position</td>
<td>€27.5M</td>
</tr>
</tbody>
</table>

**Dual listing**

Euronext Paris & Nasdaq Copenhagen - Ticker: ONXEO
Multiple near to mid-term R&D value-creating milestones

**platON™ platform**
*From 2018 on*
New compounds and their potential applications

**AsiDNA™ (IV)**
*End 2017*
Filing for a Phase I as monotherapy in solid tumors
*Early 2018*
Results of ongoing in-vivo preclinical studies in other tumors/combinations
*From H2 2018 on*
Intermediate findings from phase I/II combo

**belinostat**
*Early 2018*
Results of ongoing preclinical studies in other tumors/combinations
*2018*
Initiation of a phase I/II study in combination (IV) and/or monotherapy (oral)
*From H2 2018 onwards*
Intermediate findings from phase I/II combo
Onxeo: Advancing innovation towards breakthrough cancer therapies

- A clear value creating strategy by driving innovative programs to best inflexion points to generate deals
- R&D programs based on 2 of the most promising mechanisms of action, with multiple development paths and wide potential of applications
- A sound translational expertise to drive optimal compounds’ development
- A proven capacity to generate transactions and enrich the pipeline
Contacts
Judith Greciet – CEO
Nicolas Fellmann – CFO
Tel: +33 1 45 58 76 00
contact@onxeo.com

Company Information
www.onxeo.com
International board of industry experts

JUDITH GRECIET
CEO

DANIELLE GUYOT-CAPARROS
Independent director

CHRISTINE GARNIER
Independent director

ELVIRA SANZ
Independent director

JOSEPH ZAKRZEWSKI
Chairman of the Board

JEAN-PIERRE BIZZARI
Independent director

THOMAS HOFSTAETTER
Independent director

JEAN-PIERRE KINET
Independent director

NICOLAS TREBOUTA
Director, representing Financière de la Montagne

November 2017
AsiDNA™ demonstrates strong synergy in combination with HDAC inhibitors
AsiDNA™: Strong synergy in combination with PARPi*
(In vitro studies on tumor cell lines)

- Synergistic effect of AsiDNA™ combined with various PARP inhibitors
  - Increased unrepaird DNA break sites, DNA damages and cell lethality in different tumor cell lines
  - No lethality observed in healthy cells

- Cytotoxic synergistic effect obtained with Talazoparib on two tumor cell lines (solid and liquid) without any cytotoxicity on healthy cells

*PARPi: inhibitors of the enzyme poly ADP ribose polymerase (PARP)
AsiDNA™: Strong synergy in combination with HDACi
(In vitro studies on tumor cell lines)

- Strong synergistic effect between AsiDNA™ and histone deacetylase inhibitors (HDACi)
  - The synergy remains over time after repeated administration: potential for new treatment schemes
  - Similar results obtained with other HDACi such as vorinostat, entinostat, romidepsin

- Strong cytotoxic synergistic effect observed with belinostat on both solid and liquid tumor cell lines, without any cytotoxicity on healthy cells

Source: preclinical results; please refer to the Company’s press release on September 28, 2017


Inhibition of DNA damage repair by artificial activation of PARP with siDNA. Croset A, Cordelières FP, Berthault N, Buhler C, Sun JS, Quanz M, Dutreix M. Nucleic Acids Res. 2013, 41:7344-55.


Chemosensitization of hepatocellular carcinoma by the novel DNA repair inhibitor DT01 in mouse and rabbit models. Herath N, et al., Submitted - European radiology (under review)


ASCO 2015 annual meeting. Abstract #143029.


Safe and Effective Treatment of Patients with Peripheral T-cell Lymphoma (PTCL) with the Novel HDAC Inhibitor, Belinostat, in Combination with CHOP: Results of the Bel-CHOP Phase 1 Trial. (Johnston, P., et al., ASH 2015).

A Phase II trial of Belinostat (PXD101) in patients with relapsed or refractory peripheral or cutaneous T-cell lymphoma. (Foss, F., et al., 2014, BJH)


Phase II activity of belinostat (PXD-101), carboplatin, and paclitaxel in women with previously treated ovarian cancer. (Dizon, D.S, et al., Int J Gynecol Cancer, 2012)

A Phase I/II Clinical Trial of Belinostat (PXD101) in Combination with Doxorubicin in Patients with Soft Tissue Sarcoma. (Vitfell-Rasmussen, J., et al., 2016, Sarcoma)

Paclitaxel/Carboplatin With or Without Belinostat as Empiric First-Line Treatment for Patients With Carcinoma of Unknown Primary Site: A Randomized, Phase 2 Trial. (Hainsworth, J.D., et al., 2015, Cancer, 121(10), 1654-61).
Key lessons from ReLive

Study did not meet its primary endpoint
No survival improvement versus the comparative group.

As single agent, similar effect as BSC
BSC included other anticancer agents (incl. oxaliplatin, gemcitabine or tyrosine kinase inhibitors, mono & combos).

Unexpected high survival in the BSC group

Favorable overall safety and tolerability profile
Including in those patients who underwent the longest treatment periods, beyond 12 months.
Perspectives for Livatag®

Licensing opportunities to be explored after full understanding of the data

“The Relive study did not meet its primary endpoint, partly due to the high survival rate in the control arm, which was unprecedented except in the most recent phase III negative trial post sorafenib in HCC. However, Livatag tends to show a similar level of efficacy as recently reported for regorafenib* in second line, in a well preserved liver function population (Child–Pugh A), although both drugs cannot be compared due to the lack of assessment of both drugs in the same trial.”

Prof. Philippe Merle, MD, Professor in Hepatology (La Croix Rousse Hospital, Lyon, France) and Coordinating Investigator of the ReLive study.

Onxeo’s press release on September 11, 2017

- Reallocation of resources to R&D programs with a focus on DNA-targeting technologies, AsiDNA™ and belinostat, and combos to generate short and mid terms value catalysts

Potential for development in combinations?

Potential for development in Asia?

Potential for development in earlier BCLC stage?

*Livatag was not tested head-to-head in a clinical trial with regorafenib which was used in the ReLive control arm for only a few patients.
Livatag®: Publications

- Phase I clinical trial and pharmacokinetic evaluation of doxorubicin carried by polyisohexylcyanoacrylate nanoparticles. (Kattan, J. et al. Investigational New Drugs 10: 191-199, 1992.)
- A randomized phase II trial of Doxorubicin-Transdrug™ (Livatag®) demonstrates significant overall survival increase in inoperable hepatocellular carcinoma (HCC) patients. Merle, P. et al. 2011 (In Book of abstracts)
- Dose and infusion rates drive lung toxicity of Doxorubicin Transdrug (DT) in rats. Merle, P. et al. 2014 (In Book of abstracts)
- Mechanistic study of the relative cytotoxicity of doxorubicin loaded nanoparticle formulation compared to free doxorubicin in hepatocellular carcinoma (HCC) cell lines. Trochon-Joseph, V et al. AACR 2016, Abstr nr 2143
- Safety and efficacy results from the phase 3 relive study of doxorubicinloaded nanoparticles versus best standard of care in patients with advanced hepatocellular carcinoma after failure or intolerance to previous treatment including sorafenib, P. Merle et al, (O-020)

(Summary of key findings on Onxeo’s website - http://www.onxeo.com/pdf/170918_Onxeo_Support_Slides_Call_Relive.pdf)