Onxeo announces development of Beleodaq® oral formulation opening new opportunities for its HDAC inhibitor

The company has obtained the first set of positive results of PK study with new oral formulation of Beleodaq®

Paris (France), Copenhagen (Denmark), June 2, 2016 – Onxeo S.A. (Euronext Paris, Nasdaq Copenhagen: ONXEO), an innovative company specializing in the development of orphan oncology therapeutics, today announced the first set of positive results from a preclinical pharmacokinetic (PK) study on the bioavailability of an oral formulation of belinostat (Beleodaq®) and the next steps in its development plan for the new formulation. Beleodaq® is a histone deacetylase inhibitor (HDACi) currently approved in the US for the treatment of patients with relapsed or refractory peripheral T-cell lymphoma (PTCL).

The current formulation of belinostat is a lyophilized formulation for intravenous (IV) use. The aim of the company is to develop an oral formulation of belinostat offering strong benefits for patients and physicians in terms of compliance, as well as opening new opportunities for indications for which an oral formulation of belinostat is relevant.

As a key step in the development plan, the company has performed a canine PK bioavailability study to screen two enabling formulation technologies dedicated to improving solubility and assessed two prototypes selected based on dissolution properties, and physical and chemical stability.

Findings from this study showed a good level of bioavailability approaching the theoretical maximum achievable as indicated by the performance of the oral solution. Onxeo will then select the best of the two formulation technologies, to pursue formulation development and obtain a suitable clinical prototype and, in parallel, fine tune the optimal dosing regimen to then start clinical development in selected indications.

Judith Greciet, CEO of Onxeo, commented: “The positive results that we have obtained represent an important milestone towards developing a clinically-ready oral formulation of belinostat, which will provide multiple benefits to patients and physicians in terms of convenience, pain-free administration, and need for assistance from medical staff, especially as the other available PTCL treatments are injectable forms. Finally, it opens up a range of new opportunities in the orphan oncology field and beyond, giving Onxeo the opportunity to extend belinostat’s patent protection, thus reinforcing the interest to evaluate the compound in combination with other drugs for new indications.”

Graham Dixon, PhD, Chief Scientific Officer of Onxeo, added: “The preclinical study conducted using the oral formulation of belinostat provides solid proof of achieving a good level of bioavailability and promising preliminary PK data. These data strongly support our next steps to conduct preclinical *in vivo* efficacy studies to confirm efficacy at achievable exposure levels and clinical trials with oral belinostat. ”

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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