Onxeo Announces Promising Results from Preclinical Study of Beleodaq® Combination with Checkpoint Inhibitors

Combination with checkpoint inhibitors decreased tumor growth rate and prolonged survival in syngeneic HCC mouse model

Paris (France), Copenhagen (Denmark), October 27, 2016 – Onxeo S.A. (Euronext Paris, Nasdaq Copenhagen: ONXEO), a biopharmaceutical company specializing in the development of innovative drugs for the treatment of orphan diseases, in particular in oncology, today announced promising results from preclinical studies evaluating the potential of its already PTCL (peripheral T-cell lymphoma)-approved Beleodaq® (belinostat), in combination with checkpoint inhibitors as a potential treatment option for other tumor indications. The studies were conducted as the second step of Onxeo's ongoing preclinical development program in partnership with leading European research institution the University of Navarra's University Clinic and Center for Applied Medical Research in Spain under the leadership of Prof. Bruno Sangro and Dr. Pablo Sarobe.

The preclinical studies were performed in an immune-competent mouse syngeneic HCC (hepatocellular carcinoma) model, in which responses to a combination of Beleodaq® and checkpoint inhibitors were compared to responses from treatment with checkpoint inhibitors alone. The studies demonstrated a complete cessation of tumor growth in all mice (100%) treated with the combination of Beleodaq® and checkpoint inhibitors. The tumor cessation effect continued for approximately one week after the final dose of belinostat was administered. By comparison, checkpoint inhibitors alone inhibited tumor growth in only about 30 percent of mice which received the treatment for the same period.

In addition, mechanistic studies performed on the spleens of the mice demonstrated the generation of an underlying immune response that correlated with the observed therapeutic effect of the combination treatment, showing an increase in production of interleukines (signaling proteins for regulating immune responses) by activated T-cells and a decrease in the number of regulatory T-cells, when compared to mice treated only with checkpoint inhibitors.

Graham Dixon, PhD, Chief Scientific Officer of Onxeo, commented, *"These data represent promising findings for the continued evaluation of Beleodaq® in cancers beyond peripheral T-cell lymphoma (PTCL) when used as a combination with immuno-oncology agents in various tumor indications such as for example HCC. Approximately 20 percent of cancer patients currently treated with checkpoint inhibitors alone are responding and thus, if translated into the clinic, the positive outcomes from the combination therapy would account for a significant improvement in the number of patients exhibiting high treatment responses. As a next step in our collaboration with Prof. Bruno Sangro and Dr. Pablo Sarobe, we are conducting follow-up studies to fully characterize these preclinical findings demonstrating the potential of Beleodaq® in combination with checkpoint inhibitors in various tumor indications, and in particular, are evaluating the immune response in the tumor microenvironment in order to improve the translation of the response into human patients."*

About Onxeo

Onxeo is a biopharmaceutical company specializing in the development of innovative drugs for the treatment of orphan diseases, in particular 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 and aggressive cancers. 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 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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