Onxeo Announces Final Data from Completed Phase II Trial of Validive® for the Prevention of Severe Oral Mucositis in Head and Neck Cancer Patients

Data confirm Validive® efficacy and safety; Provide basis for planned Phase 3 Trial
Full results to be presented at ASCO Annual Meeting 2015 on Saturday, May 30

Paris (France), Copenhagen (Denmark), May 26, 2015 – Onxeo S.A. (Euronext Paris, NASDAQ Copenhagen: ONXEO)**,** an innovative company specializing in the development of orphan oncology drugs, today announced the final data from its global Phase II clinical trial of Validive® (clonidine Lauriad®) confirming the therapy's favorable safety profile and efficacy in reducing severe oral mucositis (SOM) in patients with head and neck cancer receiving chemoradiation therapy. These positive results provide the basis for a Phase 3 trial to further evaluate the efficacy of Validive®, which Onxeo plans to initiate by end 2015.

The full study results will be presented during a poster session on Saturday, May 30 at the 2015 American Society of Clinical Oncology (ASCO) Annual Meeting, being held May 29-June 2 in Chicago, IL.

Judith Greciet, Chief Executive Officer of Onxeo, said, *"The full data from the Phase 2 trial of Validive® validate our preliminary findings from October 2014 and establish Validive® as a viable treatment option for head and neck cancer patients suffering fromSOM. We look forward to presenting these positive data at the upcoming ASCO Annual Meeting and continuing to work closely with the U.S. FDA, which granted Validive® fast-track designation in January 2014, to initiate our planned Phase 3 trial in the same patient population by the end of this year. The accelerated pathway to potential FDA approval will allow Onxeo to optimize development and review time for Validive®, and therefore enable us to bring this therapy to patients withSOM more expeditiously, which remains a high unmet medical need."*

Dr. Jordi Giralt, M.D., Ph.D., Head of the Radiation Oncology Service at Vall d'Hebron University Hospital in Barcelona, Spain, investigator of the Phase 2 trial who will present the full results at the ASCO meeting commented, "*Oral mucositis is the most frequent and debilitating complication of chemoradiotherapy in patients with head and neck cancer, and there is currently no effective intervention. With all expert members of the Advisory Board on Validive®, we are confident that these confirmed efficacy and safety findings further establish Validive® as a promising treatment option to combat the incidence and severity of SOM for these heavily-treated patients."*

Abstract 6058 (Poster 381) – Mucoadhesive clonidine (Clonidine Lauriad®) in the prevention of severe radiomucositis in head and neck cancer patients: A phase II randomized trial[1].

First author:	Jordi Giralt, M.D., Ph.D., Head of the Radiation Oncology Service at Vall d'Hebron University Hospital in Barcelona, Spain
Poster Session:	Head and Neck Cancer
Date, Time, Location:	Saturday, May 30; 1:15 - 4:45 p.m. CDT; S Hall A, McCormick Place

The completed Phase 2, multi-center, double-blind, randomized, placebo-controlled, three-arm study (NCT01385748) compared the efficacy and safety of Validive® 50 microgram (µg) and 100 µg mucoadhesive buccal tablets (MBT) to placebo in patients with head and neck cancer receiving chemoradiation therapy.

The full dataset from 183 patients (121 patients treated with Validive® and 62 patients given placebo) demonstrate that Validive® treatment reduces the incidence of SOM grade 3 or 4, with a good safety profile. While the findings were not statistically significant, all criteria - the observed differences in the incidence, in the dose of radiation before occurrence of severe mucositis and time to occurrence - were all going in the same direction, supporting the initiation of future confirmatory studies, leading the trial's Advisory Board to approve further investigation in a Phase 3 trial.

The Phase 2 study found that SOM developed in fewer patients receiving Validive® than in patients receiving placebo, 45.3% and 60.0% respectively ($p = 0.064$). Furthermore, patients receiving Validive® who developed severe oral mucositis did so at a higher median radiation dose (60.0 Gy) compared to patients receiving placebo (48.0 Gy) ($HR = 0.754$; $p = 0.211$), indicating that Validive® prevents the onset of severe oral mucositis over a wider radiation dose range.

Safety profile of Validive® was very good, with occurrence of adverse events being similar between placebo (98.4%) and Validive® groups (90.8%). However patients treated with Validive® compared to placebo experienced less nausea (49.6% vs. 71%) and dysphagia (32.8% vs. 48.4%).

References

1. Giralt, J. et al. J Clin Oncol 33:5s, 2015 (suppl; abstr 6058)

About Severe Oral Mucositis (SOM)

Severe oral mucositis is a particularly invalidating pathology occurring in more than 60% of patients treated with radio/chemotherapy for head and neck cancer and has currently no validated curative or preventive treatment. It may induce intense oral pain and eating disability requiring enteral or parenteral nutritional support. Thirty percent of patients need to be hospitalized as a result and symptoms can force patients to stop treatment for an undefined period thus reducing treatment efficacy.

About Validive®

Validive® is a therapeutic application of clonidine based on the mucoadhesive technology Lauriad®. Onxeo's proprietary Lauriad® technology significantly increases the mucous and salivary concentrations of the active ingredient it contains, with decreased systemic absorption.

As an agonist of the alpha-2 adrenergic receptors, Validive® exhibits painkilling and anti-inflammatory properties, and was developed for the prevention and treatment of chemoradioation therapy-induced severe oral mucositis in patients with head and neck cancer.

Validive® was granted orphan drug status in Europe in November 2011 and also received Fast-Track status from the U.S. Food and Drug Administration (FDA) in January 2014 for the prevention and treatment of oral mucositis induced by radiotherapy and/or chemotherapy for patients being treated for cancer.

About Onxeo

Onxeo has the vision to become a global leader and pioneer in oncology, with a focus on orphan or rare cancers, through developing innovative therapeutic alternatives designed to "make the difference". The Onxeo team is determined to develop innovative medicines that provide patients with hope and significantly improve their lives.

Key orphan oncology products at the advanced development stage are:

Livatag® (doxorubicin Transdrug™): Phase III in hepatocellular carcinoma
Validive® (clonidine Lauriad®): Phase II in severe oral mucositis: Positive preliminary top-line results
Beleodaq® (belinostat): registered in the US in peripheral T-cell lymphoma
For more information, visit the website www.onxeo.com

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