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Belinostat has activity in thymoma. Data presented at ASCO

Copenhagen, Denmark – 30 May 2009 – TopoTarget A/S (OMX: TOPO) has announced that positive data from a phase 2 study of belinostat monotherapy in patients with thymic malignancies was presented on 30 May at the ASCO 2009 conference held from 29 May – 2 June. A total of 27 patients were evaluable for response. In two out of 17 patients with thymoma a partial response was documented (13 and 13+ months), and in addition 11 patients had stable disease (4-15+ months). No response was seen in 10 patients with thymic carcinoma. The conclusion is that belinostat has activity in patients with recurrent or refractory thymoma. The thymoma cohort has been expanded to the second stage of the study and enrollment is ongoing. The study is sponsored by the Cancer Therapy Evaluation Program at the National Cancer Institute (NCI, US) under a Clinical Trials Agreement with TopoTarget for the development of belinostat.

"The data presented at ASCO concludes that belinostat has activity in patients with recurrent or refractory thymoma - a disease where there is a need for new treatment options. The thymoma cohort has been expanded to the second stage of the study" says study principal investigator Professor Giuseppe Giaccone, Branch Chief, Medical Oncology Branch, National Cancer Institute, Bethesda, MD, USA.

Thymic malignancies are rare tumors of the anterior mediastinum. Platinum-based chemotherapy is used for first-line treatment of advanced disease. About 50% of patients with advanced disease may fail initial therapy. There is no established role of second-line therapy in patients with refractory or recurrent disease. Belinostat is evaluated as treatment for recurrent thymic malignancies in the reported study based on a good pre-clinical rationale and a prolonged minor response (31 months) seen in a patient with recurrent thymoma participating in a belinostat phase I study.

The study:

Patients with recurrent thymoma or thymic carcinoma, progressing after platinum-based chemotherapy were eligible. They were required to have measurable disease, PS 0-2 and normal organ functions. Belinostat was given as a 30-minute intravenous infusion at 1000 mg/m² once daily on days 1 to 5 of a 21-day cycle until disease progression or intolerable side effects. For patients who had received more than 12 cycles of therapy, belinostat was administered every 28 days.

Results:

From December 2007 to May 2009, 32 patients with thymic malignancies have been enrolled at 2 institutions; 18 patients were males. The median age is 53.5 years (24-84), 21 thymomas and 11

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thymic carcinomas. Mean number of prior regimens was 3 (1-10). 19 patients underwent prior tumor resection and 4 had myasthenia gravis. A median of 4 cycles of belinostat has been administered (1-20+). Treatment was well tolerated, with nausea being the most common side effect and well controlled with prophylactic antiemetics. 27 patients are evaluable for response: 2 had a partial response (13, 13+ m), 15 stable disease (3-15+ m) and 10 progression. No responses were seen in 10 evaluable patients with thymic carcinomas.

Correlative markers of activity in blood and tumor were performed. Tubulin and lysine protein acetylation was generally observed in peripheral blood one hour after belinostat infusion on day 3 of the first cycle. In 9/9 patients analyzed for acetylated lysine at baseline and 1 hour post-infusion on day 3 of the first cycle, a response of between 2.2-fold and 10.4-fold over baseline was observed. In 5/5 patients analyzed at the same time points for acetylated tubulin, a response of between 2.1-fold and 8.9-fold over baseline was observed. Other correlative markers are being analyzed.

Conclusions:

Belinostat has activity in patients with recurrent or refractory thymoma. The thymoma cohort has been expanded to the second stage of the study and enrollment is ongoing.

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Background information

About belinostat

Belinostat is a promising small molecule HDAC inhibitor being investigated for its role in the treatment of a wide range of solid tumors and hematologic malignancies either as a single-agent, or in combination with other active anti-cancer agents, including carboplatin, paclitaxel, cis-retinoic acid, azacytidine and Velcade® (bortezomib) for injection. HDAC inhibitors represent a new mechanistic class of anti-cancer therapeutics that target HDAC enzymes, and have been shown to: arrest growth of cancer cells (including drug resistant subtypes); induce apoptosis, or programmed cell death; promote differentiation; inhibit angiogenesis; and sensitize cancer cells to overcome drug resistance when used in combination with other anti-cancer agents.

Intravenous belinostat is in phase III in peripheral T-cell lymphoma (PTCL) and is currently being evaluated in multiple clinical trials as a potential treatment for cutaneous and peripheral T-cell lymphomas, B-cell lymphomas, AML, mesothelioma, soft tissue sarcoma, Myelodysplastic Syndrome (MDS), and liver, colorectal, and ovarian cancers, either alone or in combination with other anti-cancer therapies. Continuous intravenous administration (CIV) is being evaluated in clinical trials in solid tumours as well as in AML. An oral formulation of belinostat is also being evaluated in a Phase I clinical trial for patients with advanced solid tumors. Several trials in the belinostat program are conducted under a Clinical Trials Agreement (CTA) under which the NCI sponsors clinical trials to investigate belinostat for the treatment of various cancers, both as a single-agent and in combination chemotherapy regimens. Furthermore TopoTarget has a Cooperative Research and Development Agreement (CRADA) with the NCI to conduct preclinical and nonclinical studies on belinostat in order to better understand its anti-tumor activity and to provide supporting information for clinical trials.

About Thymoma and Thymic carcinoma

The thymus, a small organ that lies in the upper chest under the breastbone, is part of the lymph system. It makes white blood cells, called lymphocytes, that protect the body against infections. There are different types of tumors of the thymus. Thymomas and thymic carcinomas are rare tumors (approximate incidence of 0.15 cases per 100,000) of the cells that are on the outside surface of the thymus. The tumor cells in a thymoma look similar to the normal cells of the thymus, grow slowly, and rarely spread beyond the thymus. On the other hand, the tumor cells in a thymic carcinoma look very different from the normal cells of the thymus, grow more quickly, and have

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usually spread to other parts of the body when the cancer is found. Thymic carcinoma is more difficult to treat than thymoma.

Chemotherapy is efficacious in the systemic treatment of thymomas and thymic carcinomas. Single agents have activity in the range of 10-20%, and cisplatin alone achieves 10% response rate. However combination chemotherapy achieves higher response rates; the most active combinations include cisplatin, and response rates are in the range of 40-90%. Common first line combinations include cisplatin-etoposide, cisplatin-etoposide-ifosfamide, and cisplatin-doxorubicin-cyclophosphamide, and several variations.

A large proportion of patients with advanced stages however cannot be cured with available treatments and at least 50% of these patients require second-line therapy. Several agents have been shown to have some activity in this setting. Responses seen in previously cisplatin-treated thymomas are however relatively short-lived and there is need of novel agents in this disease.

About TopoTarget

TopoTarget (OMX: TOPO) is an international biotech company headquartered in Denmark, dedicated to finding "Answers for Cancer" and developing improved cancer therapies. The company was founded and is run by clinical cancer specialists and combines years of hands-on clinical experience with in-depth understanding of the molecular mechanisms of cancer.

TopoTarget has a broad clinical pipeline but is currently focusing on the development of belinostat, which has shown proof of concept as monotherapy in treating haematological malignancies and positive results in solid tumours where it can be used in combination with full doses of chemotherapy, and is in phase III in PTCL. TopoTarget's expertise in translational research is utilizing its highly predictive in vivo and in vitro cancer models. TopoTarget is directing its efforts on key cancer targets including HDACi, NAD+, mTOR, FasLigand and topoisomerase II inhibitors. The company's first marketed product Savene[®]/Totect[®] was approved by EMEA in 2006 and the FDA in 2007 and is marketed by TopoTarget's own sales force in Europe and the US. For more information, please refer to www.topotarget.com.

TopoTarget Safe Harbour Statement

This announcement may contain forward-looking statements, including statements about our expectations of the progression of our preclinical and clinical pipeline including the timing for commencement and completion of clinical trials and with respect to cash burn guidance. Such statements are based on management's current expectations and are subject to a number of risks and uncertainties that could cause actual results to differ materially from those described in the forward-looking statements. TopoTarget cautions investors that there can be no assurance that actual results or business conditions will not differ materially from those projected or suggested in such forward-looking statements as a result of various factors, including, but not limited to, the following: The risk that any one or more of the drug development programs of TopoTarget will not proceed as planned for technical, scientific or commercial reasons or due to patient enrolment issues or based on new information from non-clinical or clinical studies or from other sources; the success of competing products and technologies; technological uncertainty and product development risks; uncertainty of additional funding; TopoTarget's history of incurring losses and the uncertainty of achieving profitability; TopoTarget's stage of development as a biopharmaceutical company; government regulation; patent infringement claims against TopoTarget's products, processes and technologies; the ability to protect TopoTarget's patents and proprietary rights; uncertainties relating to commercialization rights; and product liability expo-sure; We disclaim any intention or obligation to update or revise any forward-looking statements, whether as a result of new information, future events, or otherwise, unless required by law.