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Further results of the phase II clinical trial with belinostat in cancer of unknown primary (CUP) indicate clinical activity

Copenhagen, Denmark – August 28, 2012 – Topotarget A/S (NASDAQ OMX: TOPO) today announced further exploratory analysis for its randomized phase II belinostat trial in patients with CUP.

Following the release of the top-line results on June 29, 2012, Topotarget has further reviewed and analyzed the outcome of the CUP phase II study with support from investigators and members of our Global Oncology Advisory Board.

- As previously communicated, the study did not meet its primary endpoint of improving the median progression-free survival (PFS), but showed a highly statistically significant increase (43.2% vs. 22.2%; p=0.0252) in objective response rate in the intent-to-treat (ITT) population. In addition, the median overall survival (OS) in the ITT population was 11.5 months for the belinostat and carboplatin/paclitaxel (BelCaP) treated group versus 9.1 months for patients on carboplatin/paclitaxel (CaP), a 2.4 months increase in OS favoring the BelCaP group (hazard ratio (HR)=0.861; p=0.2906, non-significant).

- Topotarget has carried out additional analyses of the underlying data including a "time to failure (TTF) of treatment" assessment where patients who went on subsequent therapy prior to disease progression were taken into account. Failure of treatment is defined as death, progression of the malignant disease, or addition of new anti-cancer therapy prior to disease progression (whichever came first). The analysis was carried out as a substantial number of patients (six in all; five patients in the CaP group and one patient in the BelCaP group received new and additional anti-cancer therapy even though a progression had not been documented at the time of adding therapy. This TTF analysis showed an increase in the median duration to failure of treatment of 2.2 months in favor of BelCaP compared to CaP (5.4 months in the BelCaP group versus 3.2 months in the CaP group in the per-protocol (PP) population; HR was 0.892 [0.540-1.473] (p=0.3271, non-significant). Furthermore, the OS in the PP population showed a 30% survival improvement trending towards statistical significance (HR 0.771, p=0.1744).

- The TTF data as well as the OS results, albeit being not statistically significant, together with the impressive overall response rate (ORR) indicate clinical activity of the BelCaP regimen in the treatment of patients with CUP.

"Taken together, we believe that there are interesting signals of clinical activity when using belinostat in combination with standard chemo regimen (CaP – carboplatin and paclitaxel) in the treatment of solid tumors," said CEO, Francois Martelet. "Further randomized and sufficiently powered studies in well-defined disease entities such as lung, bladder, and ovarian cancer using belinostat in combination with CaP chemotherapy regimen may confirm these preliminary clinical signals. In addition, and separate from the CUP study, we, later this year, look forward to the results of our currently on-going pivotal BELIEF study in the hematologic indication of peripheral T-cell lymphoma using single agent belinostat".

Trial background
The CLN-17 study in CUP was a randomized, controlled, open-label, multinational, comparative efficacy and safety phase II trial of Topotarget's compound belinostat in combination with carboplatin and paclitaxel (BelCaP), compared to carboplatin and paclitaxel (CaP), in patients with previously untreated CUP.

The objective of the trial was to provide an estimate of the hazard ratio of treatment effect of BelCaP compared to CaP, with the primary study endpoint being defined as a PFS improvement of at least 60%. Secondary endpoints include overall response rate (ORR), OS, and safety assessment.

The main findings from the study were:

- The **primary efficacy analysis of PFS** for BelCaP vs. CaP in the ITT population did not show statistical significance at the 10% level for the stratified, one-sided log-rank test. The p-value was 0.5526 and the median PFS was 5.4 versus 5.3 months. HR = 1.034 [95% confidence interval (CI): 0.631, 1.694]

- The **response rate in the ITT population** was 43.2% vs. 22.2%, which is statistically significant. HR = 2.850 [CI: 1.117, 7.272], p-value = 0.0252

- The **median OS in the ITT population** was 9.1 on CaP versus 11.5 on BelCaP, a 2.4 months increase in OS favoring the BelCaP group. HR = 0.861 [CI: 0.504, 1.469], p-value = 0.2906, non-significant

- The **median OS in the PP population** was 9.1 on CaP versus 11.5 on BelCaP, a 2.4 months increase in OS favoring the BelCaP group. HR = 0.771 [CI: 0.447, 1.330], p-value = 0.1744, non-significant. PP was defined as patient population who received assigned treatment and with post-baseline tumour assessment and no major protocol violation

- The **BelCaP combination** was generally well-tolerated during the first six cycles of treatment. This was also the case in the subsequent maintenance period where belinostat was given as oral monotherapy treatment

- Finally we also evaluated if the histology, the degree of differentiation, or the site of metastasis had any bearing on the doubling of the response rate. The outcome of the read-out indicates that these parameters do not significantly impact the outcome of the primary analysis

Conclusion
The study's primary endpoint of PFS was not met although there was a highly significant increase in the response rate.

When taking patients who received additional therapy prior to progression of disease into account, the analysis did show a non-significant, but clinically interesting, increase

in time to treatment failure in the BelCaP group by >2 months in the PP analysis. The median OS was increased by >2 months in the BelCaP group (not significant).

We conclude that this study provides encouraging signs of clinical efficacy and a favorable safety profile of belinostat when used in combination with carboplatin/paclitaxel.

We are working together with key investigators and expect data to be presented at major international oncology meetings in 2013. We also plan on submitting the study results for peer-review in international oncology journals.

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Background information

About Topotarget
Topotarget (NASDAQ OMX: TOPO) is an international biopharmaceutical company headquartered in Copenhagen, Denmark, dedicated to clinical development and registration of oncology products. In collaboration with Spectrum Pharmaceuticals, Inc., Topotarget focuses on the development in pivotal studies of its lead drug candidate, belinostat, which has shown positive results as a monotherapy treating hematological malignancies and positive results in solid tumors. Belinostat may be used in combination with full doses of chemotherapy, and is in a pivotal trial within PTCL (peripheral T-cell lymphoma). For more information, please refer to www.topotarget.com.

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