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Top-line data announced for clinical belinostat trial in cancer of unknown primary (CUP)

Copenhagen, Denmark – June 29, 2012 – Topotarget A/S (NASDAQ OMX: TOPO) today announced top-line results for its randomized phase II belinostat trial in patients with cancer of unknown primary (CUP).

Data from the primary analysis of the on-going phase II trial in CUP showed that the difference in progression-free survival (PFS) did not meet the protocol-specified primary endpoint. However, the objective response rate (ORR), one of the secondary endpoints, showed statistically significant benefit for the BelCaP arm in the intent to treat (ITT) analysis.

Trial results
A total of 89 patients have been recruited at 23 sites throughout Europe and the US. The patient population was well-balanced between the two arms. Based on the ITT analysis, the primary PFS endpoint did not meet statistical significance. The analysis of secondary endpoints showed statistically significant difference in ORR (BelCaP 43.2% versus CaP 22.2%, *p*-value of 0.025). Responses occurred earlier in the BelCaP arm. The evaluation of overall survival (OS) indicates a separation of the survival curves after approximately eight months with a benefit for the BelCaP arm. The triple combination of belinostat and CaP was well-tolerated.

Trial background
The CLN-17 study in CUP is a randomized, controlled, open-label, multinational, comparative efficacy and safety phase II trial of Topotarget's compound belinostat (PXD101) in combination with carboplatin and paclitaxel (BelCaP), compared to carboplatin and paclitaxel (CaP), in patients with previously untreated CUP.

The objective of the trial is 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 objective response rate (ORR), overall survival (OS), and safety assessment.

Conclusion
"There are no approved drugs for the treatment of patients with CUP and there remains a high unmet medical need. Despite the fact that the study did not meet the primary efficacy endpoint of PFS we do see encouraging clinical signals for time to response and response rate, which may translate into an improvement in overall survival seen on this trial. Maintenance treatment with oral belinostat was generally well-tolerated allowing for prolonged treatment past 6 cycles of the BelCaP combination. Further analysis is on-going to evaluate subsets of patients that may benefit from belinostat treatment. We continue to believe in the potential of belinostat,

used either as monotherapy or in combination with other treatments. We remain committed to the clinical development of belinostat in both solid tumors and hematologic malignancies", says Francois Martelet, CEO of Topotarget.

Conference call

Topotarget will host a conference call today, June 29, 2012, at 11.30 am (CET) at which the executive management will present and highlight the announced CUP top-line data. The presentation will be held in English. A slideshow will be available on Topotarget's website, www.topotarget.com, at the start of the conference call.

To participate in the conference call, please dial:

From Denmark: +45 70 26 50 40 or +45 70 27 90 09

Outside Denmark: +44 208 817 9301

A replay of the conference call will be available approximately three hours after the conference call and until August 29, 2012 at: +45 70 25 26 01 or +35 314 364 267, pass code: 7834 305#. A replay of the conference call will also be available on Topotarget's website www.topotarget.com.

Topotarget A/S

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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.

Topotarget Safe Harbor 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 enrollment issues or based on

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