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Belinostat pivotal BELIEF trial meets primary endpoint

Topotarget A/S today announced that the primary endpoint has been met for the belinostat pivotal trial for patients with peripheral T-cell lymphoma (PTCL).

The pivotal BELIEF trial (PXD101-CLN-19) evaluating the efficacy and safety of belinostat for the treatment of patients with relapsed/refractory PTCL met its primary endpoint. A Special Protocol Assessment (SPA) agreement with the U.S. Food and Drug Administration (FDA) requires the BELIEF trial to reach an objective response rate (ORR) of at least 20% and this objective was met. Data from the trial are being further analyzed and are expected to be communicated during Q4 2012.

Under the terms of a 2010 license and collaboration agreement, belinostat is currently being developed jointly by Spectrum Pharmaceuticals and Topotarget. Spectrum possesses the commercial rights to market belinostat in North America and India. A New Drug Application (NDA) for belinostat in PTCL is expected to be filed with the FDA by Spectrum in H1 2013.

Should Spectrum receive FDA's acceptance to file the belinostat NDA, Topotarget is entitled to receive one million shares of common stock in Spectrum and a double-digit million USD cash payment. If belinostat is approved by the FDA, Topotarget will further receive a double-digit million USD cash payment.

BELIEF is a pivotal, open-label, multi-center, single-arm efficacy and safety trial of i.v. belinostat in patients with relapsed or refractory PTCL. FDA has granted belinostat Orphan Drug and Fast Track designation for the treatment of PTCL. The trial was initiated in December 2008 and recruitment was completed with 129 patients in September 2011. In total, the study included approximately 100 clinical centers globally.

PTCL is a hematological disease including a heterogeneous group of malignancies of T-cell origin that represents approximately 10-15% of all cases of non-Hodgkin's lymphoma. PTCL is an aggressive, high-grade type of cancer with a poor prognosis of expected average survival of approximately two years from diagnosis without treatment. The projections for annual cancer incidences point to 15,500 new cases of PTCL in the US, Japan, and five largest EU countries.

Belinostat is a novel pan-HDAC inhibitor in late-stage clinical development with more than 1,000 patients treated. Belinostat has a promising safety profile, which allows

combination with traditional chemotherapy. Preclinical experiments demonstrated belinostat to be effective against multiple cancers by inhibiting cell proliferation and inducing programed cell death (apoptosis) in tumor cells. Belinostat has been tested in a number of phase I/II clinical trials in hematological cancers and solid tumors both in mono- and combination therapy. Data from these trials have provided evidence of the anti-tumor effect of belinostat, including as monotherapy in PTCL and cutaneous T-cell lymphoma (CTCL), liver cancer, and thymoma.

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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 of its lead drug candidate, belinostat, which has shown positive results in the treatment of hematological malignancies and solid tumors, obtained by both mono- and combination therapy. For more information, please refer to www.topotarget.com.

Forward-looking 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 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 exposure. 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.