



To NASDAQ OMX Copenhagen A/S
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Topotarget confirms receipt of acceptance-to-file milestone payment

Topotarget A/S hereby confirms the receipt of the milestone payment of USD 10 million and 1 million shares from Spectrum Pharmaceuticals, Inc. related to the US Food and Drug Administration's (FDA) recent acceptance to file Beleodaq™ (belinostat) for peripheral T-cell lymphoma (PTCL).

Received milestone payment

On February 6, 2014, Topotarget announced that the FDA had granted acceptance to file the New Drug Application (NDA) for Beleodaq for the treatment of relapsed or refractory PTCL. The acceptance to file triggered a milestone payment to Topotarget of USD 10 million and 1 million shares, with a current value of approximately USD 8 million, from US partner Spectrum Pharmaceuticals. The USD 10 million has been received and the Spectrum Pharmaceuticals shares are expected to be sold within a non-disclosed time period.

Priority Review of Beleodaq

The FDA has assessed that Beleodaq may potentially cover an unmet medical need in the treatment of patients with relapsed or refractory PTCL and thereby holds the potential to provide a significant advance in medical care. Beleodaq has therefore been granted Priority Review. When a drug is designated Priority Review the review process is expected to be six months from the acceptance to file date (announced on February 6, 2014) compared to the standard 10 months.

The FDA has assigned an action date of August 9, 2014. By or on this date, we expect to receive feedback from the FDA regarding an approval of the Beleodaq NDA.

Next milestone in 2014

An additional milestone cash payment of USD 25 million from Spectrum Pharmaceuticals is triggered upon an NDA approval. Upon an approval, Topotarget will moreover be eligible to receive potential royalty payments and sales milestones.

Unchanged outlook

This company announcement does not change the financial outlook for 2014 compared to that communicated on February 6, 2014.

Topotarget A/S

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Background information

About Topotarget

Topotarget (NASDAQ OMX: TOPO) is a Scandinavian-based biopharmaceutical company headquartered in Copenhagen, Denmark, dedicated to the clinical development and registration of oncology products. In collaboration with Spectrum Pharmaceuticals, Inc., Topotarget focuses on the development of its lead drug candidate, Beleodaq™, 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.

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About Beleodaq

Beleodaq is a novel pan-HDAC (histone deacetylase) inhibitor with more than 1,100 patients treated. Beleodaq has a favorable safety profile which may allow combination with traditional chemotherapy. Preclinical experiments showed that Beleodaq may be effective against multiple cancers by inhibiting cell proliferation and inducing programmed cell death (apoptosis) in tumor cells. Beleodaq 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 Beleodaq, including as monotherapy in PTCL, cutaneous T-cell lymphoma (CTCL), and liver cancer, and as combination therapy in soft tissue sarcoma (STS) and thymoma.

About the BELIEF study

The pivotal study of Beleodaq for the treatment of R/R PTCL was closed for recruitment in September 2011 after the inclusion of 129 patients. Final data presented at the American Society for Clinical Oncology (ASCO) annual meeting in 2013 showed an objective response rate (ORR) of 26% in all PTCL patients, 28% in PTCL patients with platelet counts above 100,000/ μ L, and 45.5% in patients with the PTCL subtype angioimmunoblastic T-cell lymphoma (AITL). Safety data presented at the T-Cell Lymphoma Forum in 2013 showed a favorable safety profile of Beleodaq when compared to the approved treatments for patients with PTCL and it was emphasized that combining Beleodaq with cytotoxic regimens is likely feasible. Beleodaq appears to have low myelosuppression and even patients with a poor bone marrow reserve tolerated Beleodaq.

About the BelCHOP study

The dose-finding BelCHOP study is designed to determine what dose of Beleodaq combined with CHOP can be safely administered together for first-line treatment of patients with PTCL. The purpose is furthermore to establish the recommended dose for the immediate following phase III confirmatory study. The dose-finding study of BelCHOP is expected to recruit up to 28 patients by Q4 2014. The confirmatory phase III study is expected to be initiated in H1 2015.

Topotarget Safe Harbor Statement

This announcement may contain forward-looking statements, including statements about Topotarget A/S' expectations to the progression of Topotarget A/S' clinical pipeline and with respect to cash burn guidance. Such statements are subject to risks and uncertainties of which many are outside the control of Topotarget A/S, and which could cause actual results to differ materially from those described. Topotarget A/S disclaims 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 Danish law.