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TopoTarget A/S

Symbion Fruebjergvej 3 DK 2100 Copenhagen Ø

Denmark

Tel: +45 39 17 83 92 Fax: +45 39 17 94 92 CVR-nr: 25695771

www.topotarget.com

# TopoTarget Provides Update on Savicol™ Pivotal Phase II Study for the Treatment of FAP and Results from Two Pharmacokinetic (PK) Studies

Patient enrolment progressing
Pharmacokinetic (PK) studies show treatment safe and well-tolerated

Copenhagen, Denmark – December 19, 2007 – TopoTarget A/S (OMX: TOPO) today announced an update on the status of the Savicol™ pivotal Phase II study for the treatment of FAP, Familial Adenomatous Polyposis (an inherited pre-disposition to develop colon cancer where standard treatment is removal of the colon). Forty six patients out of a planned 66 patients have now been enrolled and 26 patients have completed the first 6 months treatment. Thirteen patients have completed the optional 6-months extension phase.

The study is a blinded, randomized, placebo-controlled multi-center study carried out in Germany, Denmark and Russia with two study arms, one where patients receive Savicol  $^{\text{TM}}$  - a novel, proprietary formulation of the HDAC-inhibitor (HDACi) valproic acid (VPA) - and the other group receiving placebo. After 6 months of treatment in one of these groups, the patients may enter into an extension treatment period of another 6 months using active drug treatment only.

Recruitment is ongoing, with so far 46 out of a planned 66 patients included. Of this group, 26 have completed the first 6 months of treatment. The efficacy evaluation at the end of the initial 6-month treatment period includes an endoscopic examination of the colon, assessment of polyp numbers and the clinical appearance of the colon, and the analysis of biopsy material collected for specific pathological investigations, including the analysis of colorectal biomarkers.

# Pharmacokinetics (PK) characteristics of the new and proprietary formulation of Savicol™ show the treatment to be safe and well-tolerated

Two clinical PK studies with 36 healthy volunteers have been completed. The absolute bioavailability of oral Savicol™ after fasting and with concomitant food intake in comparison to an intravenously applied comparator was close to 100 %. Furthermore, serum level values of Savicol™ were recorded to follow a dose proportional pattern at the investigated doses. In addition, a linear pharmacokinetics for the examined dose range was observed. The single and multiple doses of Savicol™ applied were in general well-tolerated.

"We hope that Savicol $^{\text{TM}}$  could provide a much needed treatment for this serious condition" said Dr. Peter Buhl Jensen, CEO of TopoTarget. "The PK



#### TopoTarget update on the pivotal Phase II study of Savicol™

studies show that TopoTarget's formulation is safe and well-tolerated by patients."

Following the recent grant of US patent rights, protecting the use of Savicol<sup>TM</sup> for use in treating a range of cancer indications, TopoTarget is also currently considering the possibility of expanding the development of Savicol<sup>TM</sup> into selected cancer indications.

Today's news does not change TopoTarget's full-year financial guidance for 2007.

# TopoTarget A/S

For further information, please contact:

Dr. Peter Buhl Jensen	Telephone	+45 39 17 83 41
Chief Executive Officer	Mobile	+45 21 60 89 22
Ulla Hald Buhl	Telephone	+45 39 17 83 92
Director IR & Communications	Mobile	+45 21 70 10 49

#### **Background information**

## About Savicol™

Savicol<sup>TM</sup> (formerly referred to as PEAC®) is based on a novel and proprietary, orally available formulation of valproic acid (VPA). VPA is a molecule which acts as an HDAC inhibitor, preferentially for HDAC class I isoenzymes, which are involved in excessive cell proliferation and tumourigenesis. Furthermore, the Savicol<sup>TM</sup> formulation allows a specific pharmacokinetic release pattern of valproic acid expected to effectively inhibit these target enzymes. The HDAC inhibitory effect of VPA has already been demonstrated in Phase I trials on the basis of biomarker monitoring of histone acetylation.

Savicol  $^{\text{TM}}$  was granted Orphan Drug status for the FAP indication in Europe in 2004 and in the US in 2005.

## About TopoTarget

TopoTarget (OMX: TOPO) is a biotech company, headquartered in Denmark and with subsidiaries in the US, Switzerland, Germany and the UK, dedicated to finding "Answers for Cancer" and developing improved cancer therapies. TopoTarget is founded and run by clinical cancer specialists and combines years of hands-on clinical experience with in-depth understanding of the molecular mechanisms of cancer. Focus lies on highly predictive cancer models and key cancer targets (including HDACi, NAD+, mTOR, FasLigand and topoisomerase II inhibitors) and a strong development foundation has been built. TopoTarget has a broad portfolio of small molecule preclinical drug candidates and nine drugs (both small molecules and protein based) are in clinical development, including both novel anti-cancer therapeutics and new cancer indications for existing drugs. Savene™/Totect™ were approved by EMEA in 2006 and the FDA in 2007 and is TopoTarget's first product on the market. For more information, please refer to <a href="https://www.topotarget.com">www.topotarget.com</a>.

#### **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



#### TopoTarget update on the pivotal Phase II study of Savicol™

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.

