

A photograph of a man in a white shirt carrying a young girl on his shoulders. They are standing on a beach with waves crashing in the background. The man is smiling and looking towards the right. The girl is also smiling and waving her hand.

Interim report

for the period
January 1 – March 31, 2014



topotarget

Highlights

Highlights from Q1 2014

- February 6, 2014: The US Food and Drug Administration (FDA) grants Acceptance to File and Priority Review for Beleodaq™ (belinostat) New Drug Application (NDA) in peripheral T-cell lymphoma (PTCL)
- March 26, 2014: Topotarget confirms receipt of acceptance-to-file milestone payment of USD 10 million and 1 million shares from Spectrum Pharmaceuticals

Highlights after Q1 2014

- April 16, 2014: Topotarget and BioAlliance Pharma enter into merger agreement to create a leading orphan oncology company

Highlights from the financial results for the period January 1 - March 31, 2014

- Topotarget recognized revenues of DKK 98.2 million during the period (DKK 0.4 million in the same period in 2013)
- The research and development costs were DKK 4.3 million during the period (DKK 6.1 million in the same period in 2013)
- The administrative expenses were DKK 3.3 million during the period (DKK 3.8 million in the same period in 2013)
- Non-recurring items were DKK 5.8 million during the period (DKK 0.0 million in the same period in 2013)
- The net financials were a net loss of DKK 1.8 million during the period (net loss of DKK 0.4 million in the same period in 2013)
- The net profit from continued operations before tax for the period was DKK 82.8 million (net loss of DKK 10.2 million for the same period in 2013)
- Total comprehensive income for the period was a net profit of DKK 75.3 million (net loss of DKK 10.2 million for the same period in 2013)
- The Group's net cash and cash equivalents as of March 31, 2014 totaled DKK 109.7 million (DKK 31.5 million at year-end 2013)

Financial Calendar 2014

Event	Date
Extraordinary General Meeting	June 27, 2014
Interim report for the period January 1 - June 30, 2014	August 14, 2014
Interim report for the period January 1 - September 30, 2014	November 6, 2014

Financial highlights

Financial highlights and ratios	Q1 2014	Q1 2013	Total 2013
DKK '000			
Revenues	98,240	403	8,338
Research and development costs	(4,319)	(6,108)	(23,019)
Administrative expenses	(3,270)	(3,813)	(18,406)
Non-recurring items	(5,829)	-	-
Operating profit/(loss)	84,615	(9,822)	(34,148)
Net financials	(1,788)	(356)	(2,045)
Net profit/(loss) from continued operations before tax	82,827	(10,178)	(36,193)
Net profit from discontinued operations	-	-	-
Total comprehensive income for the period	75,327	(10,178)	(34,968)
Basic and diluted EPS continued operations	0.53	(0.08)	(0.25)
Fully diluted EPS continued operations	0.51	-	-
Basic and diluted EPS continued and discontinued operations	0.53	(0.08)	(0.25)
Fully diluted EPS continued and discontinued operations	0.51	-	-
Consolidated balance sheet			
Cash and cash equivalents	109,707	53,794	31,483
Equity	318,793	266,667	243,092
Total assets	343,000	290,659	265,117
Consolidated cash flow statement			
Cash flow from operating activities	78,245	(14,240)	(35,623)
Cash flow from investing activities	(21)	74	152
Cash flow from financing activities	-	26,500	25,494
Consolidated ratios			
Number of fully paid shares at the end of period	143,317,114	143,294,620	143,317,114
Average number of shares for the period	143,316,364	133,716,307	140,916,162
Assets/equity	1.1	1.1	1.1
Market price at the end of period	3.25	3.00	2.98
Net asset value per share	2.22	1.86	1.70
Average number of full-time employees	12	13	13

Management report

2014 has so far brought three truly epoch-making events for Topotarget, two of which occurred in Q1 2014.

- In February, Topotarget achieved Acceptance to File and Priority Review with the FDA for belinostat for the treatment of PTCL, with a Prescription Drug User Fee Act (PDUFA) action of August 9, 2014. This event triggered a milestone payment from our partner Spectrum Pharmaceuticals of USD 10 million and 1 million share; a very positive and favorable start to 2014.
- In March, we announced Topotarget's updated vision, mission, and strategy. The vision being to be a leading orphan oncology company, with the mission of being a biopharmaceutical company focused on the development of novel and innovative therapies within rare cancer diseases, providing hope for patients and their families, increasing life expectancy and quality of life. The strategy to achieving the vision is divided in four main pillars: 1. Explore belinostat opportunities, 2. Prepare for commercialization, 3. Pursue new product opportunities, and 4. Actively pursue potential M&A activities with companies who share our vision.
- In April, Topotarget entered into a merger agreement with BioAlliance Pharma to create a leading orphan oncology company. With this merger, the two companies aim at creating a highly complementary pipeline of late-stage products addressing significant unmet medical needs.

Merger with BioAlliance Pharma

The merger agreement between Topotarget and BioAlliance Pharma was entered into

on April 16, 2014 with the objective of creating a leading orphan oncology company with a broad pipeline consisting of late-stage products for the treatment of rare, or orphan, cancer diseases.

The need for new treatment options for orphan diseases is extensive. The total market for orphan oncology drugs exceeded approximately USD 45 billion in 2013 and is expected to reach approximately USD 80 billion in 2018. The overall unmet medical need is significant and new orphan diseases are continuously discovered.

The new entity will be supported by a highly complementary pipeline of late-stage products with several significant value-creating events anticipated in both the short and medium term, and will benefit from operational efficiencies, combined knowledge-sharing, and a diversified revenue stream driving growth.

The merger agreement has been unanimously approved by the Boards of Directors of both companies and is endorsed by the two largest institutional shareholders of both companies – in the case of BioAlliance Pharma by Financière de la Montagne and Idinvest Partners, representing 18.8% of the share capital of BioAlliance Pharma and in the case of Topotarget by HealthCap funds and HBM Healthcare Investments, representing 12.6% of the non-diluted share capital of Topotarget – who have agreed to vote in favor of the merger proposal at the upcoming extraordinary

“2014 has so far brought three truly epoch-making events for Topotarget”.
CEO, Anders Vadsholt

general meetings of the respective companies. We will call for an Extraordinary General Meeting (EGM) on June 27, 2014 to request the shareholders' votes for the proposed merger.

Outlook 2014

Given the initiated process of the merger plan, the Board of Directors does not believe it to be prudent to provide an outlook on earnings for 2014 until the EGM have provided their vote in respect of the contemplated merger. The company will continue to report the company's performance until the merger plan has been approved. The most important milestone for the company this year is still the expected approval of the NDA anticipated by August 9, 2014. This would trigger a milestone payment of USD 25 million and moreover the company would be eligible to receive potential royalty payments and sales milestones going forward.

Looking forward

When looking forward, Topotarget's future appears bright and promising. We look forward to the month of June in which it will be determined whether Topotarget and BioAlliance can unite to create a leading orphan oncology champion. And we look forward to the month of August by when we most likely will know if belinostat has achieved marketing approval for PTCL in the USA. Eventful months are still to come and we look forward to embracing them.

Events in Q1 2014

Acceptance to file

On February 6, 2014, Topotarget announced that the FDA had granted acceptance to file and Priority Review for the Beleodaq™ (belinostat) NDA for the treatment of relapsed or refractory PTCL. The FDA decision date based on the PDUFA is set for August 9, 2014. The acceptance to file entailed a milestone payment of USD 10 million and 1 million shares in Spectrum Pharmaceuticals, Inc. to Topotarget.

Receipt of milestone

On March 26, 2014, Topotarget confirmed the receipt of the milestone payment of USD 10 million and 1 million shares from Spectrum Pharmaceuticals, Inc. related to the FDA's acceptance to file Beleodaq™ (belinostat) for PTCL.

Events after Q1 2014

Merger agreement with BioAlliance Pharma

On April 16, 2014, Topotarget entered into a merger agreement with BioAlliance Pharma with a view to create a leading orphan oncology company with a highly complementary pipeline of late-stage products addressing significant unmet medical needs within the orphan oncology area. Please refer to Company Announcement 06-14 released on April 16, 2014.

Pipeline update

BELINOSTAT KEY CLINICAL STUDIES

Indication	Study	Sponsor	Phase I	Phase II	Pivotal	NDA	Target #	Enrollment status	Milestone	Time frame
PTCL	BELIEF (CLN-19)	SPPI*)	→				100	Completed	NDA approval	Q3 2014
PTCL	BelCHOP SPI-Bel-12-104	SPPI	→				28	Recruiting	Recruitment completed	Q4 2014
NSCLC	SPI-1014-Bel	SPPI	→				35	Completed	Recruitment completed	-
Mass balance study	SPI-12-103	SPPI	→				6	Completed	Recruitment completed	-

*) Spectrum Pharmaceuticals

NCI-sponsored studies	Initiated
Belinostat for Solid Tumors and Lymphomas in Patients with Varying Degrees of Hepatic Dysfunction	Q4 2010
A Phase I Study of Belinostat in Combination With Cisplatin and Etoposide in Adults with Small Cell Lung Carcinoma	Q2 2009

Peripheral T-cell lymphoma (PTCL) – BELIEF (CLN-19)

Acceptance to file and Priority Review granted by the FDA with a Prescription Drug User Fee Act (PDUFA) action date of August 9, 2014.

The pivotal study of belinostat for the treatment of R/R PTCL was closed for recruitment in September 2011 after the inclusion of 129 patients. Final top-line data presented at the American Society of Clinical Oncology Annual Meeting 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 January 2013 showed a favorable safety profile of belinostat when compared to the

approved treatments for patients with PTCL and it was emphasized that combining belinostat with cytotoxic regimens is likely feasible. Belinostat appears to have low myelosuppression and even PTCL patients with a poor bone marrow reserve tolerate belinostat.

BelCHOP – SPI-Bel-12-104

The dose-finding BelCHOP (belinostat plus cyclophosphamide, hydroxydaunorubicin, oncovin, and prednisone) study is designed to determine what dose of belinostat combined with CHOP can be safely administered together for the 1st-line treatment of patients with PTCL. The purpose is furthermore to establish the recommended dose for the immediate following phase III confirmatory study as agreed with the FDA. The dose-finding study of BelCHOP is expected to recruit up to 28 patients by Q4 2014. The confirma-

tory phase III trial is expected to be initiated in H1 2015.

Non-small cell lung cancer (NSCLC) – SPI-1014

This is a phase I/II maximum tolerated dose study of belinostat in combination with carboplatin and paclitaxel (BelCaP) in chemotherapy-naïve patients with stage IV NSCLC. The study was initiated in March 2011 and all patients have been enrolled for the study. Topotarget and Spectrum Pharmaceuticals are cosponsors and Spectrum Pharmaceuticals is overlooking the US-based study.

Mass balance study – SPI-12-103

This is a phase I study for the evaluation of excretion (mass balance) and pharmacokinetics of ¹⁴C-labeled belinostat in patients with recurrent or progressive malignancy. It is a supportive study for further under-

standing of belinostat's metabolism and excretion. The recruitment of six evaluable patients has been completed and the analysis of the biologic samples is ongoing.

NCI-sponsored studies

The National Cancer Institute (NCI) is a prestigious, world-leading oncology research organization sponsoring a vast

number of studies in oncology. In collaboration with Topotarget and Spectrum Pharmaceuticals, the NCI studies belinostat and investigates treatment options in indications with a high unmet medical need. The NCI sponsors and conducts the studies under their auspices and therefore the timelines and communication given are under the control of the NCI.

Financial review

The financial report for Q1 2014 comprises the parent company Topotarget A/S and the three wholly-owned subsidiaries Topotarget UK Ltd, Topotarget Germany AG, and Topotarget Switzerland S.A. (the Group).

Unless otherwise stated, the financial review is based on the Group's consolidated financial information for the period January 1 to March 31, 2014 with comparative figures for the Group for the same period in 2013.

Income statement

The total comprehensive income for the period was a net profit of DKK 75.3 million (a net loss of DKK 10.2 million for the same period in 2013).

Revenues

Topotarget recognized revenues of DKK 98.2 million during the period (DKK 0.4 million in the same period in 2013). Revenues are composed of income as

per our collaboration agreement with Spectrum Pharmaceuticals and the received milestone payment of USD 10 million and 1 million shares from Spectrum Pharmaceuticals.

Costs

Production costs, which amounted to DKK 0.2 million (DKK 0.3 million in the same period in 2013), include Topotarget personnel costs related to the Spectrum Pharmaceuticals collaboration agreement.

Research and development costs were DKK 4.3 million (DKK 6.1 million in the same period in 2013). The reduction in costs by DKK 1.8 million, or 29%, is primarily due to the near completion of most clinical projects.

Administrative expenses amounted to DKK 3.3 million (DKK 3.8 million in the same period in 2013); a decrease in costs by DKK 0.5 or 14%.

Non-recurring items amounted to DKK 5.8 million (DKK 0.0 in the same period in 2013) and relates to incentive payments, a strategic review of the company, and the one-time costs related to the merger agreement entered into between Topotarget and BioAlliance.

Net financials

The net financials showed a net expense of DKK 1.8 million (DKK 0.4 million net expense in the same period in 2013). The financial expense is mainly due to exchange rate fluctuations in foreign currencies.

Balance sheet

The balance sheet amounted to DKK 343.0 million in total assets as of March 31, 2014 (DKK 265.1 million at year-end 2013). The Group's net cash and cash equivalents as of March 31, 2014 totaled DKK 109.7 million (DKK 31.5 million at year-end 2013) and the equity amounted to DKK 318.8 million (DKK 243.1 million at year-end 2013).

Statement by the Board of Directors and Executive Management

The Board of Directors and Executive Management today discussed and adopted the interim report for Topotarget A/S for the period January 1 to March 31, 2014.

The interim report is presented in accordance with International Accounting Standards, IAS 34, as adopted by the EU and additional Danish disclosure requirements for presentation of interim reports of listed companies.

The interim report is not audited or reviewed.

In our opinion, we consider the applied accounting policies to be appropriate and adequate for the interim report. Furthermore, the interim report in our opinion gives a true and fair view of the Group's assets, liabilities, and financial position at March 31, 2014 and of the results of the Group's operations and cash flow for the

period January 1 to March 31, 2014. We also believe that the management commentary contains a fair view of the development in the Group's financial position as a whole together with a description of the principal risks and uncertainties that they face.

Copenhagen, May 8, 2014

Executive Management

Anders Vadsholt
CEO

Board of Directors

Bo Jesper Hansen
Chairman

Anker Lundemose

Gisela Schwab

Ingelise Saunders

Jeffrey H. Buchalter

Karsten Witt

Per Samuelsson

Consolidated statement of comprehensive income for the period

	Q1 2014	Q1 2013	Total 2013
DKK '000			
Revenues	98,240	403	8,338
Production costs	(207)	(304)	(1,061)
Research and development costs	(4,319)	(6,108)	(23,019)
Administrative expenses	(3,270)	(3,813)	(18,406)
Non-recurring items	(5,829)	-	-
Operating loss	84,615	(9,822)	(34,148)
Financial income and expenses	(1,788)	(356)	(2,045)
Profit/(loss) from continued operations before tax	82,827	(10,178)	(36,193)
Tax on profit for the period	(7,500)	-	1,225
Net profit/(loss) from continued operations	75,327	(10,178)	(34,968)
Net loss from discontinued operations	-	-	-
Total comprehensive income for the period	75,327	(10,178)	(34,968)
Total comprehensive income attributable to:			
Owners of the company	75,327	(10,178)	(34,968)
Non-controlling interests	-	-	-
Total comprehensive income for the period	75,327	(10,178)	(34,968)
Basic and diluted EPS continued operations	0.53	(0.08)	(0.25)
Fully diluted EPS continued operations	0.51	-	-
Basic and diluted EPS continued and discontinued operations	0.53	(0.08)	(0.25)
Fully diluted EPS continued and discontinued operations	0.51	-	-
Average number of employees	12	13	13

Condensed balance sheet

	March 31, 2014	March 31, 2013	Total 2013
DKK '000			
Assets			
Intangible assets	228,282	228,902	228,282
Tangible assets	594	2,128	784
Non-current investments	366	427	359
Non-current assets	229,242	231,457	229,425
Receivables	4,051	5,408	4,209
Cash and cash equivalents	109,707	53,794	31,483
Current assets	113,758	59,202	35,692
Assets	343,000	290,659	265,117
Equity and liabilities			
Equity	318,793	266,667	243,092
Non-current liabilities	-	3,334	-
Current liabilities	24,207	20,658	22,025
Liabilities	24,207	23,992	22,025
Equity and liabilities	343,000	290,659	265,117

Condensed cash flow statement

	March 31, 2014	March 31, 2013	Total 2013
DKK '000			
Operating profit/(loss)	84,615	(9,822)	(34,148)
Reversal of share-based payments	374	149	1,319
Depreciation, amortization, and impairment losses	205	527	1,861
Working capital changes	(5,159)	(1,755)	(5,287)
Cash flow from operating activities before interest	80,035	(10,901)	(36,255)
Received and paid interest etc.	(1,790)	(3,339)	(618)
Refunded income taxes	-	-	1,250
Cash flow from operating activities	78,245	(14,240)	(35,623)
Purchase of tangible assets	(14)	-	-
Sale of tangible assets	-	-	10
Purchase of investments	-	74	-
Repayment to non-current investments	(7)	-	142
Cash flow from investing activities	(21)	74	152
Proceeds from issuance of shares	-	26,500	25,494
Cash flow from financing activities	-	26,500	25,494
Increase/decrease in cash and cash equivalents	78,224	12,334	(9,977)
Cash and cash equivalents at January 1	31,483	41,460	41,460
Cash and cash equivalents at March 31	109,707	53,794	31,483
Total cash and cash equivalents at March 31	109,707	53,794	31,483

Consolidated statement of changes in equity for the period January 1 – March 31, 2014

	Number of shares	Share capital	Share premium account	Retained earnings	Total
		DKK '000	DKK '000	DKK '000	DKK '000
Equity at January 1, 2014	143,317,114	143,317	34,495	65,280	243,092
Net profit for the period	-	-	-	75,327	75,327
Total comprehensive income for the period	-	-	-	75,327	75,327
Recognition of share-based payment	-	-	374	-	374
Reversal of expired warrants	-	-	-	-	-
Issuance of shares	-	-	-	-	-
Costs related to capital increases	-	-	-	-	-
Share capital increase through warrant exercise	-	-	-	-	-
Equity at March 31, 2014	143,317,114	143,317	34,869	140,607	318,793

The share capital is an undistributable reserve, while the other reserves are distributable for dividend purposes subject to the provisions of the Danish Public Companies Act.

Consolidated statement of changes in equity for the period
January 1 to March 31, 2013:

Equity at January 1, 2013	132,652,050	132,652	33,849	84,746	251,247
Net loss for the period	-	-	-	(10,178)	(10,178)
Total comprehensive income for the period	-	-	-	(10,178)	(10,178)
Recognition of share-based payment	-	-	149	-	149
Reversal of expired warrants	-	-	(673)	673	-
Issuance of shares	10,642,564	10,643	15,857	-	26,500
Costs related to capital increases	-	-	(1,051)	-	(1,051)
Share capital increase through warrant exercise	-	-	-	-	-
Equity at March 31, 2013	143,294, 614	143,295	48,131	75,241	266,667

The share capital is an undistributable reserve, while the other reserves are distributable for dividend purposes subject to the provisions of the Danish Public Companies Act.

Note 1

Accounting policies

The interim report for the first three months of 2014 has been prepared in accordance with IAS 34 "Interim Financial Reporting" as approved by the EU and the additional Danish disclosure requirements for interim reports of listed companies. Apart from the effect of new IAS/IFRS implemented in the period, the interim report follows the same accounting policies as the financial statement for 2013. Please refer to notes 1, 2, and 29 in the financial statement for 2013 for a complete description of the Group's accounting policies.

Adoption of new or amended IAS /IFRS standards and interpretations

No new IAS/IFRS standards or interpretations with material effect on the interim report have been implemented in the first three months of 2014. Management expects no significant impact from the implementation of these new standards and interpretations in future periods either. For further information on IAS/IFRS standards and interpretations which will be implemented in 2014 or later please refer to note 1 in the financial statements for 2013.

New or amended IFRSs that have been issued but have not yet come into effect and have not been early adopted

The International Accounting Standards Board (IASB) has issued a number of new

or amended and revised accounting standards and interpretations that have not yet come into effect. Management anticipates that none of these new accounting standards and interpretations will have any significant impact on the financial statements in future periods.

Presentation currency

The interim report is presented in Danish kroner (DKK), which is the parent company's functional currency.

Management's significant accounting assumptions and estimates

Going concern

Topotarget has prepared its financial statement on a going concern basis.

Revenue recognition

Revenue is recognized when it is probable that future economic benefits will flow to the company and such economic benefits can be measured reliably. In addition, recognition requires that all significant risks and rewards of ownership of the rights or services included in the transaction have been transferred to the buyer. Income from agreements with multiple components and where the individual components cannot be separated is recognized over the period of the agreement. In addition, rec-

ognition requires that all significant risks and rewards of ownership of the goods or services included in the transaction have been transferred to the buyer. If all risks and returns have not been transferred, revenue is recognized as deferred income until all components of the transaction have been completed.

Non-recurring items

Non-recurring items include an incentive payment as a result of the Acceptance to File the NDA for belinostat with the FDA. Furthermore, costs for the strategy work and transaction costs directly related to the proposed merger between Topotarget and BioAlliance Pharma are included. The line is shown separately to facilitate the comparability of income statement and to provide a better picture of the operational result.

Financial liabilities

Included in the non-current liabilities and the current liabilities is a potential payment of USD 2.0 million to Celldex Therapeutics (formerly CuraGen) in relation to the purchase of the full belinostat rights in April 2008. The potential milestone payment will take place if and when Topotarget receive the expected milestone payment from Spectrum Pharmaceuticals or partly when the 1 million shares in Spectrum Pharmaceuticals are sold.

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