



# **Topotarget**

## **Q2 2011 interim conference call**

17 August 2011

# Safe harbour statement

This presentation 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 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 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.

# Q2 2011 activities

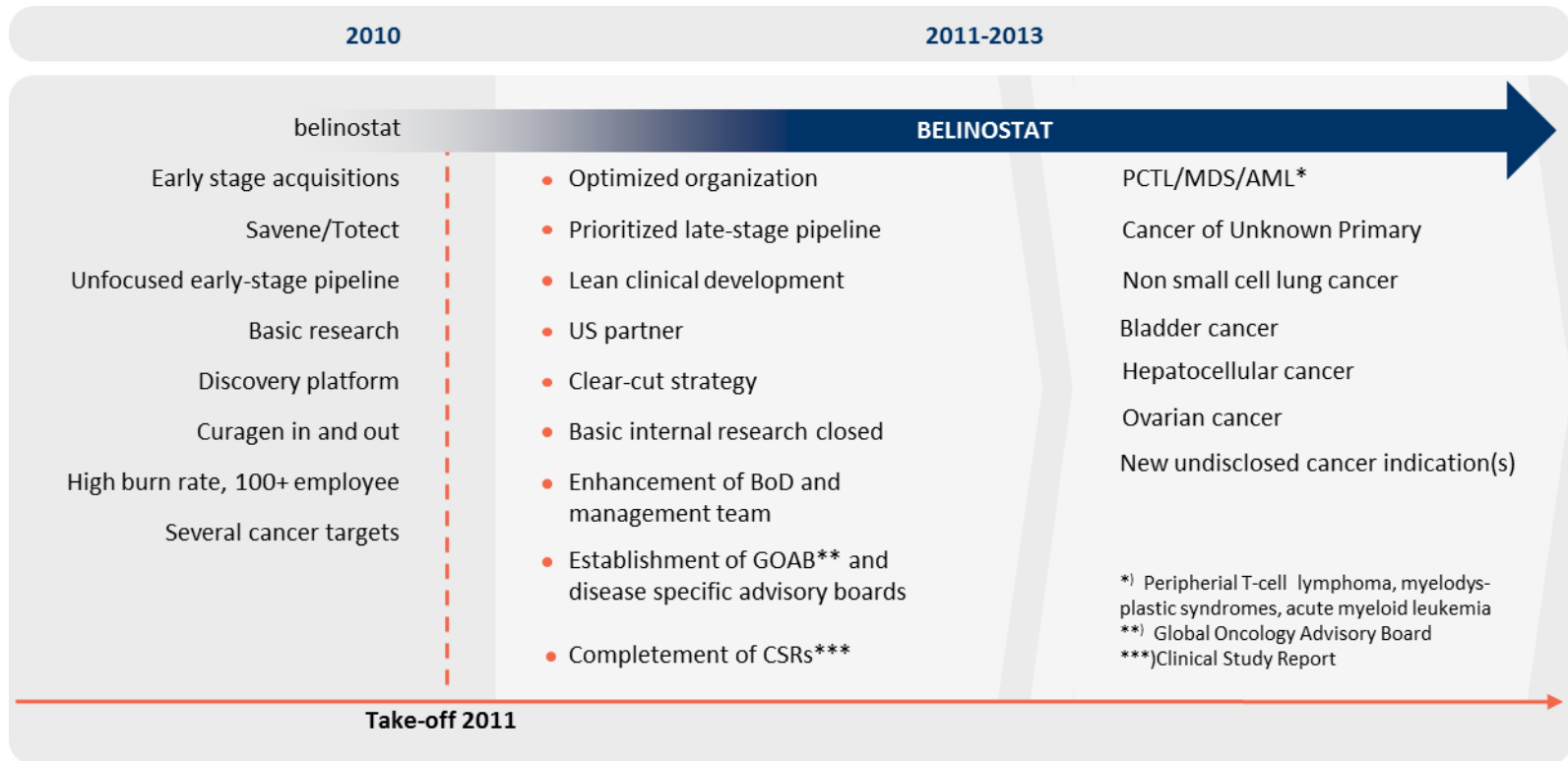
## Activities during Q2 2011

- PTCL; enrollment are ongoing and on-track
- CUP; collecting and cleaning data
- CLN-9 (oral belinostat) completes enrolling
- Completion of GOAB (Global Oncology Advisory Board)
- Belinostat abstract published at ASCO 2011

## Events after the period

- Investigator study of belinostat combined with Tarceva® in NSCLC terminated
- Proposal for the election of additional two members of Board of Directors (EGM 29 August 2011):
  - Dr. Gisela Schwap
  - Dr. Karsten Witt
- Issue of warrants to employees, management and the Board of Directors

# Focus on belinostat



# Belinostat clinical trials 2011

- Our strategy is unchanged to establish belinostat as one of the most successful HDAC-inhibitors
- We will develop belinostat in selected indications where we have reasons to believe belinostat will have efficacy in hematological malignancies and in solid tumors
- In 2011 we will continue with the clinical programs which we have already started. Enrollment in our pivotal PTCL study are expected to be completed H2 2011, and CUP topline data is expected to reported in the same period.
- We will maximize the duration of our current cash position
- New late-stage studies are rather expensive. We are continuously reviewing our options with our partner and in internationally markets (Topotarget territories)

# Belinostat key clinical trials (Topotarget and Spectrum)

Study	Sponsor	Indication	Phase I	Phase II	Pivotal	Target	Status	Milestone	Time
BELIEF	SPPI	PTCL				100-120	Recruiting	NDA Rolling Submission	2012
CLN-17	TT	CUP				89	Complete	Top-line results	H2 2011
CLN-9	TT	Solid tumor				92	Complete	Scientific publ.	H2 2011
CLN-9	TT	Lymphoma				28	Complete	Top-line results	H1 2012
CLN-14	TT	Solid + STS				55	Phase I Phase II	LPFV stage I in Phase II	H2 2011
			Results stage I	H2 2011					
CLN-20	SPPI/TT	Drug-Drug				24	Recruiting	Top-line results	H2 2011
SPI-1014 Bel	SPPI/TT	NSCLC				35	Recruiting	FPFV **)	H1 2011

\*) Last Patient First Visit \*\*) First Patient First Visit

# Commercial strategy for North America and India

## Licensing agreement with Spectrum Pharmaceuticals

- Agreement 2 February 2010
- \$30M cash upfront
- Additional value \$320M in potential milestone payments
- + Double digit royalties
- Spectrum funds PTCL BELIEF trial; Topotarget funds ongoing randomized phase II CUP study
- Resources for co-development in promising indications, cost sharing with Spectrum contributing 70% and Topotarget 30% of future development costs
- Joint development and commercialization committees
- Spectrum territory: North America and India as well as first right of negotiation to China



# Commercial goals for Topotarget territories

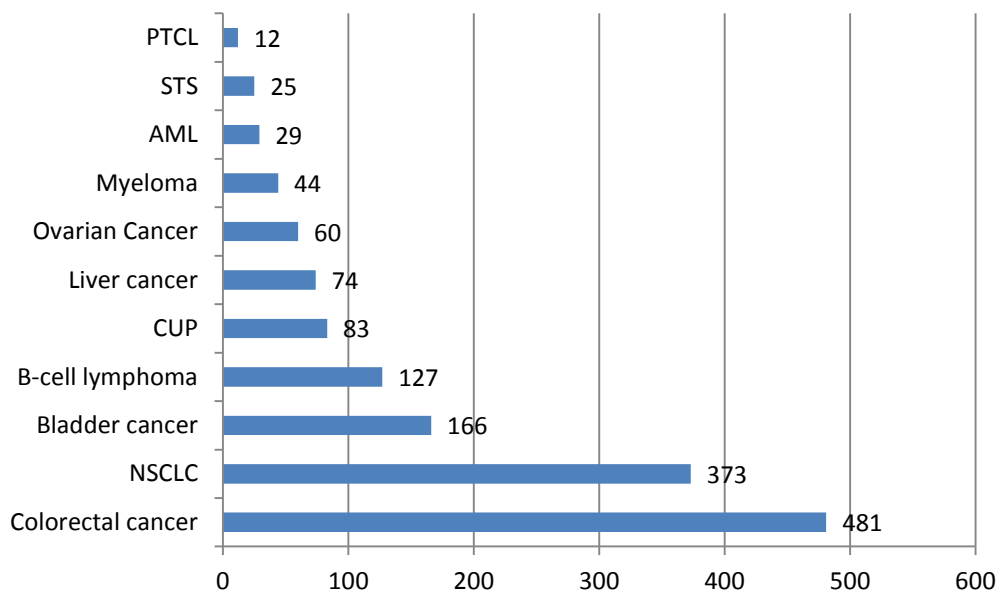
Topotarget is determined to explore the full potential of belinostat:



- Commercialize in Europe and RoW, together with a partner or by building our own market presence in selected indications.
- Expand belinostat clinical program in these regions

# Belinostat – commercial potential

## New cancer patients per year in US, Japan and 5 major EU



Source: Datamonitor estimate (US + Japan + 5 major EU)

- Belinostat has several competitive advantages with best-in-class profile:
  - Active in multiple drug resistant cancer cells
  - Mild or no bone marrow toxicity, enabling combination treatment with chemotherapy in full doses
  - The only HDACi administered as IV, CIV and oral
  - Opportunity to preselect patients with a high probability for response allows the drug to be used earlier
- Estimated annual global peak market sale of belinostat in the current PTCL indication ~ \$100-130M
- Estimated annual global market sale of the CUP indication ~ USD 0.5-0.6bn
- Price of Istodax® (romidepsin) approved November 2009 for CTCL/PTCL June 2011 is 30,000 \$\* per patient per month
- Expansion into new cancer indications will significantly add market opportunity

\*New York Times

# Achievements and goals

- Belinostat unique HDACi with both oral and IV formulation available
- > 30 clinical studies completed or ongoing
- > 900 patients being exposed to belinostat
- Good safety profile
- Ongoing registration trial in PTCL
- Randomized Phase II in CUP enrollment completed
- Substantial and untapped potential in expansion into new oncology indications
- Strong pre-clinical and emerging clinical rationale for use in treatment of several solid tumor indications with high unmet medical need
- Improved news flow in 2011

# Belinostat news flow 2011/2012

News flow						
	Indication	Design	Target accrual	Status	Milestones	Time frame
BELIEF CLN-19	PTCL	Single-arm pivotal trial with belinostat monotherapy	100-120	Recruiting	Recruitment complete NDA filing	H22011 2012
CLN-17	CUP	Randomized phase II with BelCaP versus CaP	89	Enrollment complete	Top-line results	H2 2011
CLN-9	Lymphoma	Single-arm phase I dose and schedule finding study	28	Enrollment complete	Top-line results	H1 2012
CLN-14	Solid tumors - Soft tissue sarcoma	Single-arm phase I/II dose finding study with Belinostat and doxorubicin with cohort expansion at MTD	55	Recruiting	Results of stage I in cohort expansion	H2 2011
SPI-1014-Bel	NSCLC	Single-arm phase I/II dose finding and efficacy study with BelCaP	35	Recruiting	FPFV	H1 2011

# Q2 2011 financial highlights

- Topotarget recognized revenues of DKK 62.6 million during the period (DKK 62.8 million in the same period 2010)
- A pre-tax profit of DKK 2.3 million (2010: Profit of DKK 7.6 million) was recorded for the period
- The Group's net cash and cash equivalents as of 30 June 2011 totaled DKK 151.7 million (DKK 205.1 million at year-end 2010)
- Topotarget is still expecting the pre-tax loss to be within the guidance given on 5 April 2011, corresponding to a pre-tax loss of DKK 20-40 million for 2011 and a net cash position of DKK110-130m by the end of 2011.

# Income statements

## Comprehensive income statements

All figures in DKK '000	Q2 2011	Q2 2010	6 months 2011	6 months 2010	Total 2010
Revenue	31,427	33,618	62,586	62,848	129,038
Production costs	(1,229)	(3,138)	(2,156)	(6,492)	(10,932)
Research and development costs	(13,739)	(28,501)	(29,192)	(48,026)	(71,608)
Divestiture of rights	-	(1,251)	-	32,473	32,473
Write-down of research and development projects	-	-	-	-	(189,541)
Sales and distribution costs	(3,394)	(5,393)	(6,787)	(11,488)	(19,098)
Administrative expenses	(12,244)	(12,828)	(22,260)	(21,997)	(38,778)
<b>Operating profit/(loss)</b>	<b>822</b>	<b>(17,493)</b>	<b>2,191</b>	<b>7,318</b>	<b>(168,446)</b>
Financial income and expenses	5,296	1,549	117	324	68,772
<b>Profit/(loss) before tax</b>	<b>6,118</b>	<b>(15,944)</b>	<b>2,308</b>	<b>7,642</b>	<b>(99,674)</b>
Tax on profit/(loss) for the period		-		-	43,985
<b>Net profit/(loss) for the period</b>	<b>6,118</b>	<b>(15,944)</b>	<b>2,308</b>	<b>7,642</b>	<b>(55,689)</b>
<b>Other comprehensive income</b>	-	-	-	-	-
<b>Total comprehensive income for the period</b>	<b>6,118</b>	<b>(15,944)</b>	<b>2,308</b>	<b>7,642</b>	<b>(55,689)</b>
Basic EPS (DKK)	0.05	(0.12)	0.02	0.06	(0.42)
Diluted EPS (DKK)	0.05	(0.12)	0.02	0.06	(0.42)
<b>Average number of employees</b>			<b>46</b>	<b>52</b>	<b>50</b>

# Topotarget USA, Inc.

- Revenues from Totect<sup>®</sup> sales were DKK 5.2 million in Q2 2011 (DKK 8.0 million in the same period 2010 and DKK2.4m in Q1 2011)
- Sales of Totect<sup>®</sup> during Q2 2011 in the US market were in the beginning negatively affected by wholesalers' postponement of purchases caused by a delay in the release of a new batch of Totect<sup>®</sup>
- The new batch of Totect<sup>®</sup> was released end of the second quarter
- John Parsons, Chief Commercial Officer and President of Topotarget US Inc., has resigned to pursue other opportunities with effect from July 2011. His position will be eliminated to maintain sound cost spending.

# Balance sheet

## Condensed balance sheet

All figures in DKK '000	30 June 2011	30 June 2010	Total 2010
<b>Assets</b>			
Intangible assets	235,340	426,448	235,717
Property, plant and equipment	4,341	4,709	5,991
Non-current investments	937	940	972
<b>Non-current assets</b>	<b>240,618</b>	<b>432,097</b>	<b>242,680</b>
Inventories	2,324	3,051	1,625
Receivables	19,299	21,116	16,451
Cash and cash equivalents	151,727	262,113	205,068
<b>Current assets</b>	<b>173,350</b>	<b>286,280</b>	<b>223,144</b>
<b>Assets</b>	<b>413,968</b>	<b>718,378</b>	<b>465,824</b>
<b>Equity and liabilities</b>	364,002	420,832	360,216
<b>Equity</b>	<b>364,002</b>	<b>420,832</b>	<b>360,216</b>
Non-current liabilities	14,116	136,523	14,116
Current liabilities	35,850	161,023	91,489
<b>Liabilities</b>	<b>49,966</b>	<b>297,546</b>	<b>105,605</b>
<b>Equity and liabilities</b>	<b>413,968</b>	<b>718,378</b>	<b>465,824</b>

# Cash flow

## Condensed cash flow statements

All figures in DKK '000	30 June 2011	30 June 2010	Total 2010
Operating profit/(loss)	2,192	(25,155)	(168,450)
Reversal of share-based payments	1,477	1,254	3,969
Reversal of pension commitments	-	-	(315)
Reversal of divestment of rights	-	-	(32,473)
Depreciation, amortisation and impairment losses	2,092	7,303	193,102
Working capital change	(59,186)	101,920	31,742
<b>Cash flows from operating activities before interest</b>	<b>(53,426)</b>	<b>85,322</b>	<b>27,575</b>
Received and paid interest etc.	26	9,824	12,524
<b>Cash flows from operating activities</b>	<b>(53,400)</b>	<b>95,146</b>	<b>40,099</b>
Purchase of intangible assets	-	-	-
Purchase of property, plant and equipment	65	(201)	(3,746)
Sale of property, plant and equipment	(41)	670	2,113
Purchase of investments	35	433	399
Purchase of securities	-	35,920	35,920
Sale of securities	-	-	-
<b>Cash flows from investing activities</b>	<b>59</b>	<b>36,822</b>	<b>34,686</b>
Received up-front payment belinostat	-	-	-
Proceeds from the issuance of shares	-	-	138
<b>Cash flows from financing activities</b>	<b>-</b>	<b>-</b>	<b>138</b>
<b>Increase/decrease in cash and cash equivalents</b>	<b>(53,341)</b>	<b>131,968</b>	<b>74,923</b>
Cash and cash equivalents as per 1 January 2011	205,068	130,145	130,145
<b>Cash and cash equivalents as per 30 June 2011</b>	<b>151,727</b>	<b>262,113</b>	<b>205,068</b>
<b>Cash and cash equivalents comprise:</b>			
Deposit on demand and cash	151,727	262,113	205,068
Special-term deposit	-	-	-
<b>Total</b>	<b>151,727</b>	<b>262,113</b>	<b>205,068</b>