



# Topotarget A/S

Q3 2011 Investor Presentation

November 2011

# Safe harbour statement

This presentation may contain forward-looking statements, including statements about our expectations of the progression of our pre-clinical 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.

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# Overview of belinostat clinical trials 2011-2012

Indication	Study	Sponsor	Phase I	Phase II	Pivotal	Study size	Recruitment status	Milestone	Time
PTCL	BELIEF (CLN-19)	SPPI	→			129	Completed	NDA submission	2012
CUP	CLN-17	TT	→			89	Completed	Top-line results	H1 2012
Solid tumors	CLN-9	TT	→			92	Completed	Scientific publication	2012
Lymphoma	CLN-9	TT	→			28	Completed	Top-line results	H2 2011
Solid + STS	CLN-14	TT	→	→		~55	Phase I (25) Phase II (16)	Result phase I STS expansion recruitment complete	H2 2011
Drug-drug	CLN-20	SPPI/TT	→			~39	Recruiting	n/a	n/a
NSCLC	SPI-1014 Bel	SPPI/TT	→	→		~35	Recruiting	n/a	n/a

# We are on track with the development of belinostat

## **PTCL – BELIEF study**

- Positive recommendation of the Data Monitoring Committee to the pivotal study in March 2011
- Enrollment of 129 patients successfully concluded by September 2011

## **CUP clinical study**

- Collecting and cleaning data
- Release of top-line data based on occurrence of PFS events

## **Solid tumors and relapsed/refractory lymphoma studies**

- Successful enrollment of patients into oral belinostat trial

## **Scientific presentations**

- Belinostat abstracts published at ASCO 2011, ESMO 2011, ASH 2011, amongst others



# PTCL is an orphan indication with a high unmet medical need ...

## Key facts for PTCL

- Incidence: 15,500 new cases per annum (US, Japan, and EU27)
- Prevalence: 41,000 patients (US, EU)
- Niche market: World-wide market size estimated to be USD100-130m

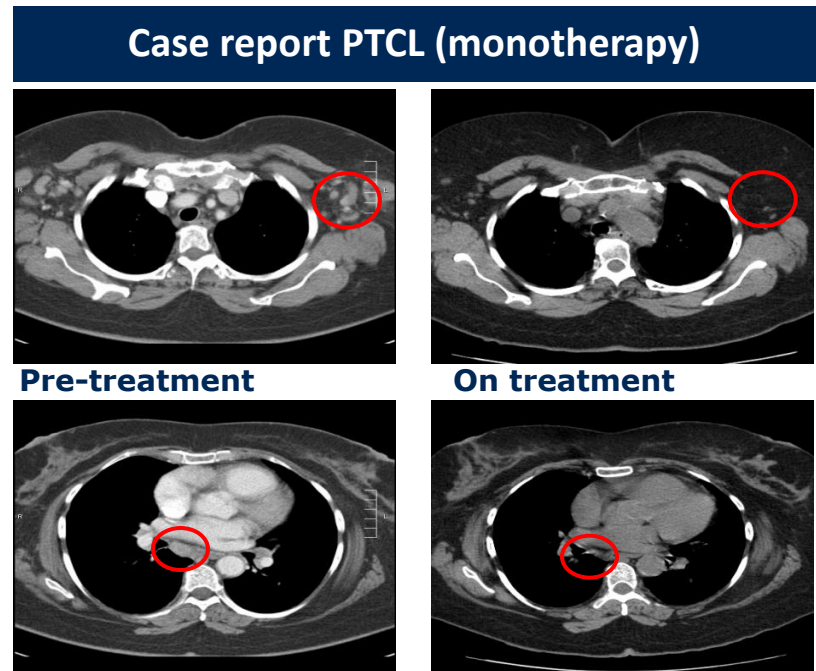
## PTCL characteristics

- Peripheral T-Cell Lymphoma (PTCL) is a subtype of non-Hodgkin's lymphoma
- 10-15% of non-Hodgkin's lymphoma patients are PTCL patients
- Aggressive, high-grade cancer
- Generally with a poor prognosis and limited treatment options
- Average age of patients with PTCL: 65 years

# ... and there are promising data from initial PTCL clinical trial

## Study CLN-6

- Phase IIa trial with patients who had refractory CTCL (28) or PTCL (25)
- Efficacy in 19 evaluable PTCL patients
  - CR: 2, PR: 4, SD: 4
  - Response rate:  
6/19 = 32% [CI: 16-45%]
  - Duration of
    - a) Response: +268 days
    - b) Stable disease: +133 days



# Financial highlights for the period January 1 to September 30, 2011

- Topotarget recognized revenues of DKK 74.3 million during the period (DKK 95.9 million in the same period in 2010)
- A pre-tax loss of DKK 14.7 million (2010: Profit of DKK 11.7 million) was recorded for the period
- The Group's net cash and cash equivalents as of September 30, 2011 totaled DKK 132.1 million (DKK 205.1 million at year-end 2010)
- Topotarget is still expecting the pre-tax loss to be within the guidance given on April 5, 2011 of DKK 20-40 million for 2011 and a net cash position of DKK 110-130 million at the end of 2011

# Comprehensive income statements for Q3 2011

All figures in DKK '000	Q3 2011	Q3 2010	9 months 2011	9 months 2010	Total 2010
Revenue	11,680	33,070	74,266	95,918	129,038
Production costs	(536)	(1,705)	(2,692)	(8,197)	(10,932)
Research and development costs	(15,305)	(14,983)	(44,497)	(63,009)	(71,608)
Divestiture of rights	-	-	-	32,473	32,473
Write down of research and development projects	-	-	-	-	(189,541)
Sales and distribution costs	(3,483)	(3,882)	(10,270)	(15,370)	(19,098)
Administrative expenses	(7,592)	(4,941)	(29,852)	(26,938)	(38,778)
<b>Operating (loss)/profit</b>	<b>(15,237)</b>	<b>7,559</b>	<b>-13,045</b>	<b>14,877</b>	<b>(168,446)</b>
Financial income and expenses	(1,746)	(3,501)	(1,629)	(3,177)	68,772
<b>(Loss)/profit before tax</b>	<b>(16,983)</b>	<b>4,058</b>	<b>(14,674)</b>	<b>11,700</b>	<b>(99,674)</b>
<b>Total comprehensive income for the period</b>	<b>(17,316)</b>	<b>4,058</b>	<b>(15,007)</b>	<b>11,700</b>	<b>(55,689)</b>
Basic EPS (DKK)	(0.13)	0.03	(0.11)	0.09	(0.42)
Diluted EPS (DKK)	(0.13)	0.03	(0.11)	0.09	(0.42)
<b>Average number of employees</b>			<b>46</b>	<b>51</b>	<b>50</b>

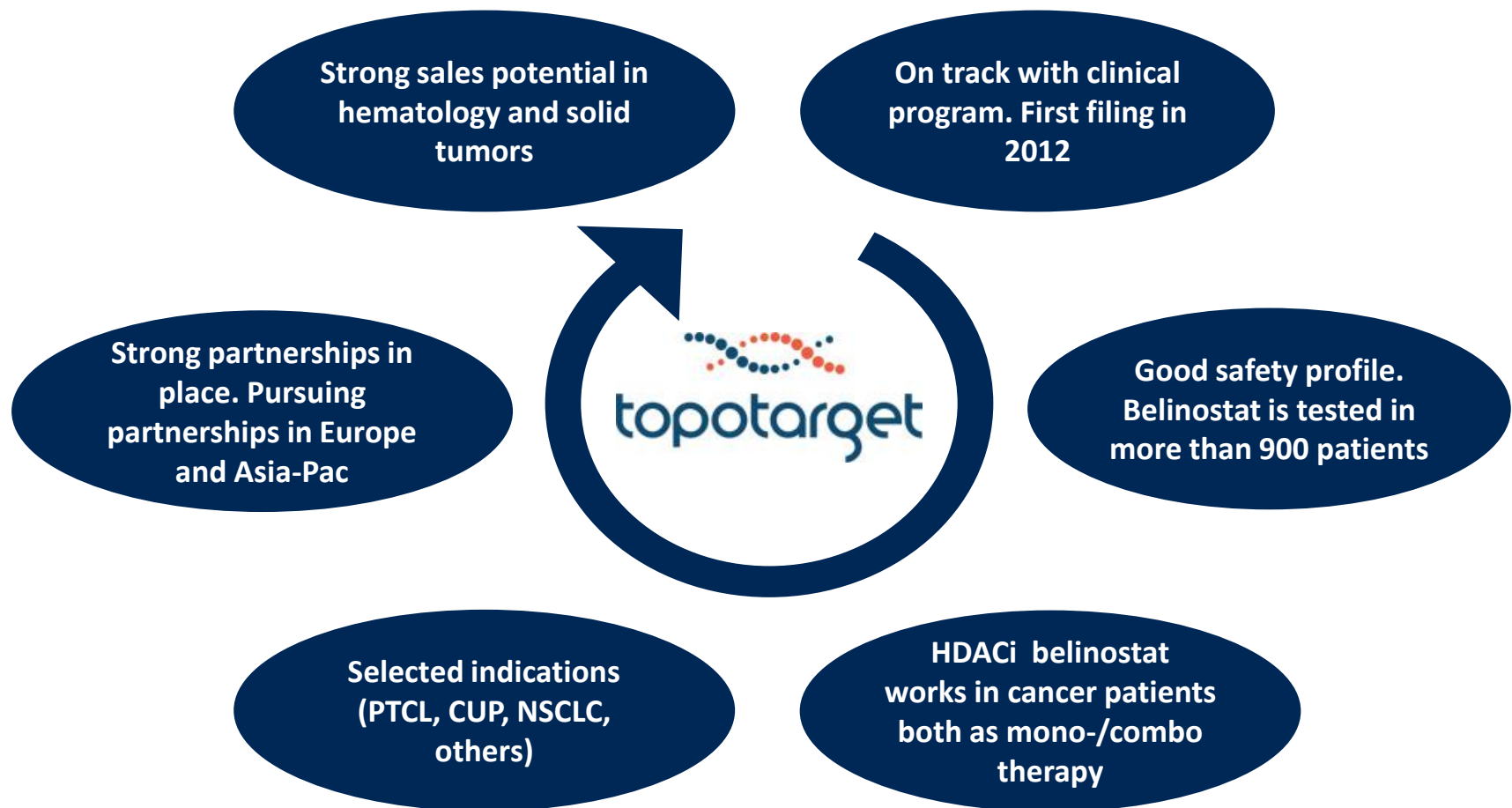
# Condensed cash flow statements 9 months 2011, key figures

All figures in DKK '000	30 Sept 2011	30 Sept 2010	Total 2010
Cash flows from operating activities before interest	(71,661)	48,929	27,575
Cash flows from operating activities	(73,044)	57,982	40,099
Cash flows from investing activities	95	37,306	34,686
Cash flows from financing activities	-	-	138
Increase/decrease in cash and cash equivalents	(72,950)	95,288	74,923
Cash and cash equivalents as per 1 January 2011	205,068	130,145	130,145
Cash and cash equivalents as per 30 September 2011	132,118	225,433	205,068
<b>Cash and cash equivalents comprise:</b>			
Deposit on demand and cash	132,118	225,433	205,068
<b>Total</b>	<b>132,118</b>	<b>225,433</b>	<b>205,068</b>

# Condensed balance sheet 9 months 2011

All figures in DKK '000	30 Sept 2011	30 Sept 2010	Total 2010
<b>Assets</b>			
Intangible assets	235,155	426,260	235,717
Property, plant and equipment	3,578	3,540	5,991
Non-current investments	795	940	972
<b>Non-current assets</b>	<b>239,527</b>	<b>430,740</b>	<b>242,680</b>
Inventories	1,374	2,674	1,625
Receivables	13,723	24,007	16,451
Cash and cash equivalents	132,118	225,433	205,068
<b>Current assets</b>	<b>147,215</b>	<b>252,114</b>	<b>223,144</b>
<b>Assets</b>	<b>386,742</b>	<b>682,854</b>	<b>465,824</b>
Equity and liabilities	347,753	425,402	360,216
<b>Equity</b>	<b>347,753</b>	<b>425,402</b>	<b>360,216</b>
Non-current liabilities	14,707	128,294	14,116
Current liabilities	24,282	129,158	91,489
<b>Liabilities</b>	<b>38,989</b>	<b>257,452</b>	<b>105,605</b>
<b>Equity and liabilities</b>	<b>386,742</b>	<b>682,854</b>	<b>465,824</b>

# Summary – building up a strong commercial profile for belinostat



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The image consists of three vertical panels, each showing a 3D ball-and-stick molecular model. The model features a central black sphere with a white sphere attached, and several red spheres connected by thin grey rods. The background is a blurred laboratory setting. In the center panel, the text "Thank you! Q&A" is overlaid in a dark blue, sans-serif font.

**Thank you!  
Q&A**