



Topotarget A/S
Corporate 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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Topotarget corporate presentation

- **Executive summary**
- Belinostat supercharging chemotherapy
- The competitive landscape of belinostat
- Financial overview

Topotarget at a glance (1)

**An international Scandinavian-based biopharmaceutical company
dedicated to develop and market cancer therapies**

- Headquartered in Copenhagen, Denmark (Medicon Valley Alliance) with ~46 employees with a lean cost structure pursued
- Focused on development and commercialization of belinostat, a molecular targeted cancer therapy
- Totect[®] indicated for anthracycline extravasation marketed by Topotarget USA, Inc.
- New management team and board in place

Listing	NASDAQ OMX Copenhagen
Symbol	TOPO.CO
Market capitalization (as of October 2011)	€ 34m
No. of shares (as of June 30, 2011)	132,652,050

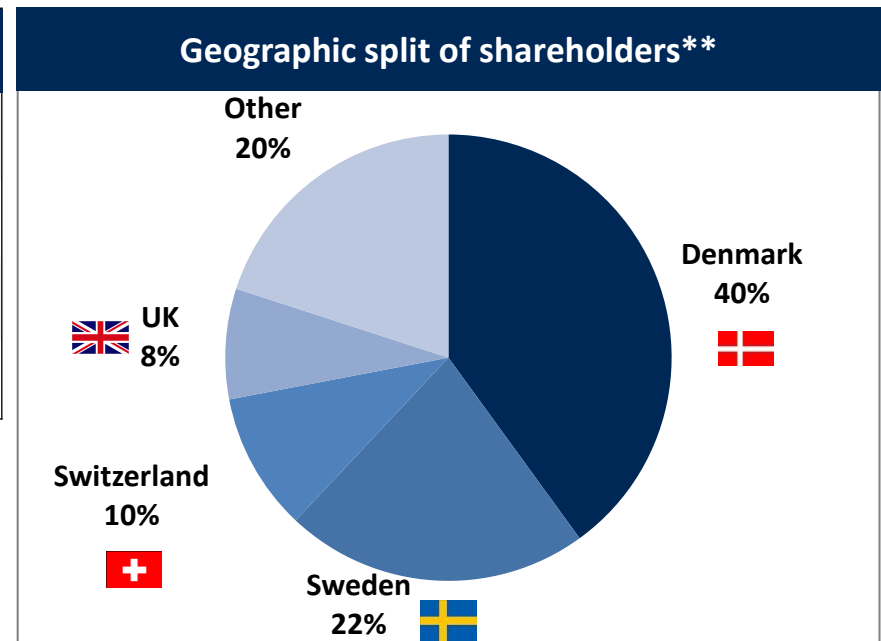
Topotarget at a glance (2)

- In collaboration with Spectrum Pharmaceuticals, Inc. (SPPI) and the NCI we are developing and commercializing belinostat in the US
- Based on current plans and without taking potential SPPI milestones into account, cash resources will take us into 2013

Geographic split of shareholders**	
The 10 largest shareholders combined*	+ 30%
HealthCap funds	+ 10%
Avanza Pension	+ 5%
3AP Fonden	+ 3%

* As of August 2011. Including HealthCap fund and excluding Avanza Pension

** Estimated



Topotarget is making a difference to cancer patients ...

Creating shareholder value

Track record

Proven track record only 7 years from idea to launch:

Totect®
Savene®

Lead development candidate

Novel cancer drug target:
HDACi

Partnerships

Strong partnerships:
Spectrum Pharmaceuticals, Inc.
National Cancer Institute
Rigshospitalet (DK)

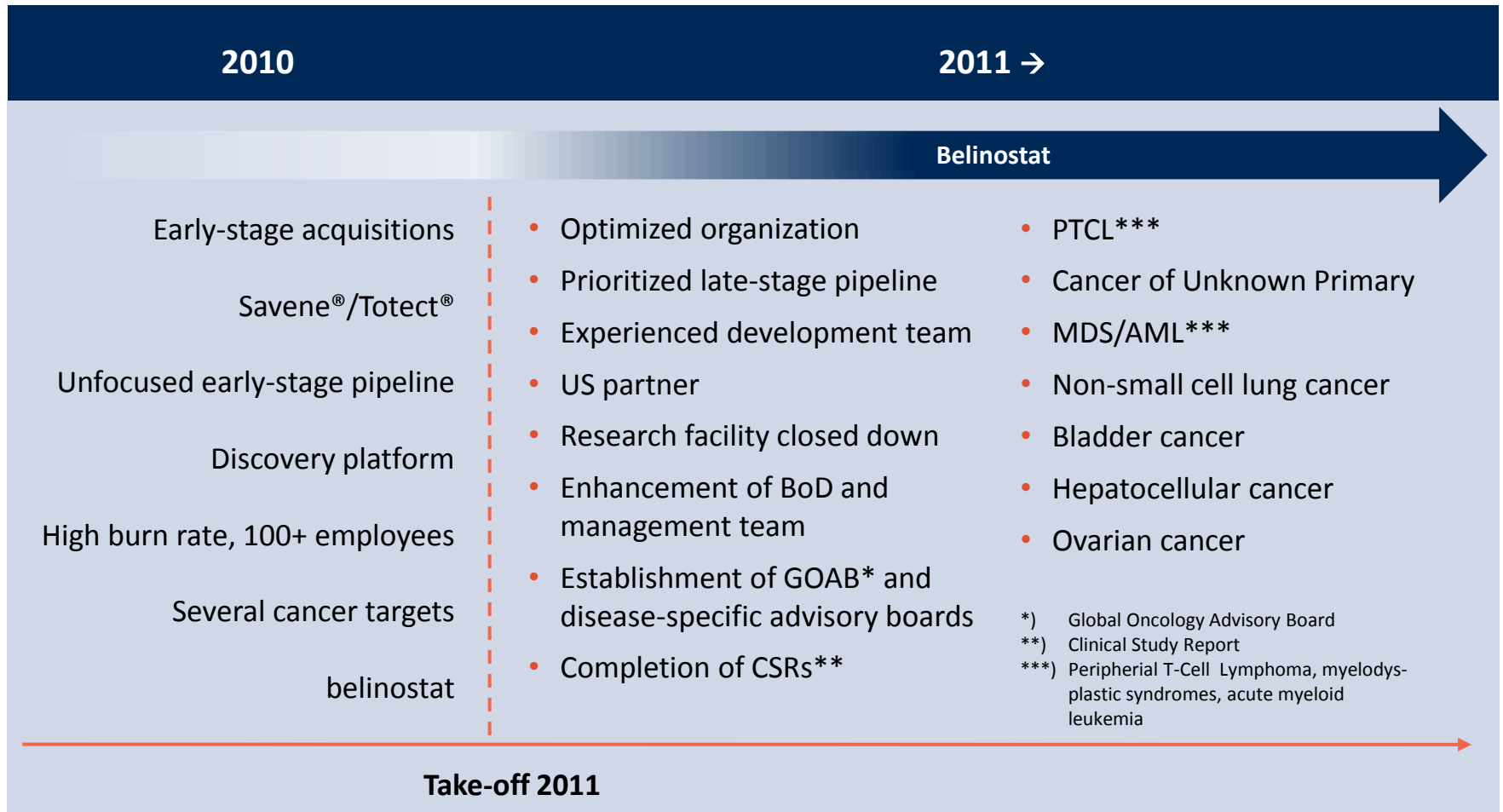
Unmet market need

Solid tumor
Hematological diseases
Solid commercial potential

Strong belinostat profile

Promising preliminary clinical efficacy
Robust safety
Strong IP
Large database
Mono- and combination therapy

... with a clear-cut focus on belinostat



Our strategy is to unlock the potential of belinostat ...

Vision

Leading Scandinavian biopharmaceutical oncology company

Mission

We are dedicated to the fight against cancer through our development and commercialization of novel cancer therapeutics

Goal

A robust business that delivers significant returns to our shareholders



How?

- Pursue NDA filing of belinostat within PTCL in the US
- EU filing will be based on an additional study
- Evaluate additional indications:
 - under the auspices of Topotarget
 - and together with our US partner
- Find partner(s) to belinostat in Europe, Asia-Pacific, and South America
- Optimize cash resources

... and we are on track to deliver!

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

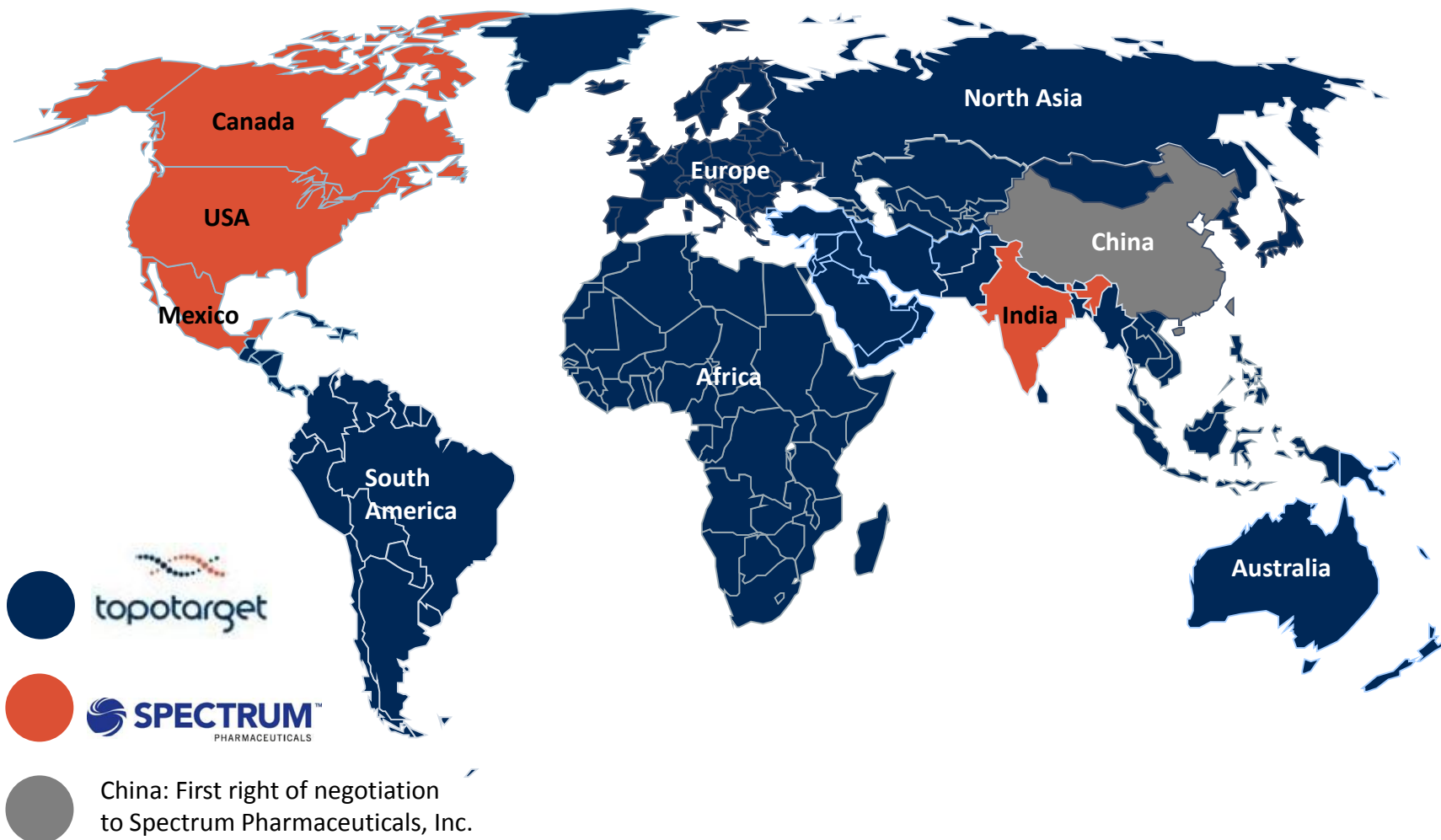
- Successful enrollment of patients into oral belinostat trial

Scientific presentations

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



Partnerships are key to a successful commercialization of belinostat



Belinostat partner agreement with



Deal terms

- Agreement, February 2, 2010
- USD 30m cash upfront
- Potential value USD 320m in milestones
- Plus double-digit royalties
- Spectrum (SPPI) funds PTCL BELIEF trial and Topotarget funds randomized phase II CUP study
- Resources for co-development in additional indications, cost sharing with SPPI contributing 70% and Topotarget 30%
- Joint development and commercialization committees set-up
- Out-license North American and Indian rights on belinostat as well as right of negotiation to China
- SPPI is responsible for submitting an NDA on belinostat for PTCL

Topotarget corporate presentation

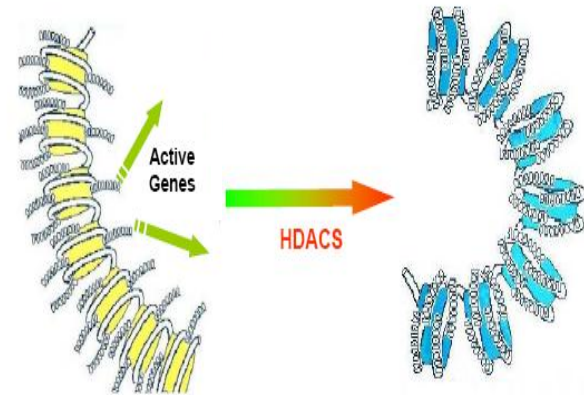
- Executive summary
- **Belinostat supercharging chemotherapy**
- The competitive landscape of belinostat
- Financial overview

Histone DeAcetylase inhibitors (HDACi)

Bypassing natural apoptosis is a hallmark of the cancer disease

Main characteristics of belinostat

- "Turns on" suppressor genes
 - Inhibiting HDACs activate silenced genes
 - Some of these are apoptotic (cell death) genes
 - Activation causes selective cancer cell death
- "Turns off" oncogenes
 - Results in inhibitions of cancer cell growth



Other mechanisms of action

- Inhibition of the growth and development of new blood vessels in effect starving cancer cells
- Induction of immune system to target cancer cells
- Interacts with for example tubulin, thus synergizing with various chemotherapies and potentially overcoming drug resistance, which is the main reason for failure of cancer treatment

Belinostat is supercharging chemotherapy

Cancer cells can over time develop resistance to the given chemotherapy – a resistance that HDACi's may overcome

HDACi's work in synergy with chemotherapy by supercharging the effects of cytostatics

Belinostat is a novel pan-HDACi with the ability of regulation of multiple class I and II HDACs

Belinostat in combination with chemotherapy increases efficacy with only a small or moderate increase in toxicity

Belinostat has a compelling clinical profile

Flexible administration

Option of multiple administration and formulation modes (IV, CIV, and oral)

Ability to combine

Combined with main established chemotherapies and by that maximizing the commercial potential

Promising efficacy data

Preliminary clinical efficacy in solid and hematological malignancies
Synergistic pre-clinical effect with established therapies

Encouraging safety profile

Shown to be well tolerated in the clinical use (≈ 900 pts.), with an excellent safety profile including cardiac toxicity profile and minimal bone marrow toxicity

Preliminary safety data confirms belinostat's differentiation potential

Belinostat preliminary safety data shows lower incidence of grade 3-4 Adverse Events within most common related AEs (nausea, fatigue, diarrhea, vomiting) when compared to marketed drugs within same class and within same primary pursued PTCL indication

	Belinostat ¹⁾	Istodax® Romidepsin ²⁾	Zolinza® Vorinostat ³⁾	Folotyn® Pralatrexate ⁴⁾
# of patients:	68	363	74	111
% of patients with grade 3-4 related Adverse Events:	%	%	%	%
Nausea	1	4	4	4
Fatigue	3	11	5	6
Diarrhea	0	2	0	2
Vomiting	0	5	0	2

Please note that for this and the following 3 slides, the comparison data is not generated in a head-to-head study. The analysis is carried out based on available published study data on the respective marketed drugs approved for CTCL and PTCL, respectively.

1) Source: Safety population from belinostat clinical studies: TT-30 and CLN-6, I.V.

2) Source: Prescribing information romidepsin, FDA label 2011-09-30, approved for CTCL and PTCL, I.V.

3) Source: Olsen et al., Phase IIb Multicenter Trial of Vorinostat ..., Journal of Clinical Oncology 2007; 25 (21), approved in CTCL, Oral

4) Source: Prescribing information pralatrexate, FDA label 2011-07-01, approved for PTCL, I.V.

... compared to pralatrexate, belinostat shows a minimum of treatment-related infections

Product	Belinostat ¹⁾		Folotyn® ²⁾ Pralatrexate	
# of patients:	68		111	
Adverse Event term:	# of related AEs	# of SAEs	# of related AEs	# of SAEs
Mucositis	2 (3%)	1	78 (70%)	>3
Sepsis	0	2	Unknown	>3
Thrombocytopenia	3 (4%)	1	45 (41%)	>3

- Toxic epidermal necrolysis has been identified post approval as an adverse reaction to pralatrexate
- Pralatrexate has been associated with severe dermatologic reactions, which may result in death. These dermatologic reactions have been reported in clinical studies (2.1% of treated patients) and post-marketing experience, and included skin exfoliation, ulceration, and toxic epidermal necrolysis

1) Source: Safety population from belinostat clinical studies : TT-30 and CLN-6, I.V.

2) Source: Prescribing information pralatrexate, FDA label 2011-07-01, approved for PTCL , I.V.

... the same pattern is observed when comparing belinostat to romidepsin

	Belinostat ¹⁾	Istodax® ²⁾ Romidepsin	
# of patients:	68	CTCL/185	PTCL/178
Adverse Event term:	# of pts. with related SAEs	# of pts. with related SAEs	# of pts. with related SAEs
Catheter-related infection	0	3	2
Cellulitis	0	Unknown	5
Central line infection	0	5	Unknown
Infection	0	Unknown	4
Pneumonia/pneumonitis	0	Unknown	9
Sepsis	0	6	7
Skin infection	0	Unknown	Unknown

- 41 out of 363 CTCL/PTCL patients (11.3%) treated with romidepsin and none of the 68 patients treated with belinostat had serious treatment-related infections
- In addition, 26 of 185 CTCL patients (14.1%) treated with romidepsin experienced dermatitis or exfoliative dermatitis related to treatment
- Of the 68 belinostat patients, 4 had skin exfoliation, 2 had skin ulcers (0 of them serious), and 1 had skin lesion, all of them unrelated to belinostat

1) Source: Safety population from belinostat clinical studies : TT-30 and CLN-6, I.V.

2) Source: Prescribing information romidepsin, FDA label 2011-09-30, approved for CTCL and PTCL, I.V.

Belinostat preliminary safety data indicates fewer related thrombocytopenia events compared to vorinostat

	Belinostat			Zolinza® Vorinostat		
	Related AEs	Grade 3 - 5	SAEs	Related AEs	Grade 3 - 5	SAEs
# of patients:		68			CTCL / 74	
Adverse Event term:						
Pulmonary embolism	0	0	0	Unknown	4	4*
Deep vein thrombosis	0	0	0	Unknown	Unknown	1*
Ischaemic stroke	0	0	0	Unknown	Unknown	1
Anaemia	2	0	1	9	1	1
Thrombocytopenia	3	2	1	16	4	1

*One case of pulmonary embolism + deep vein thrombosis

- 5 out of 74 CTCL patients (6.8%) treated with vorinostat and none of the 68 belinostat patients experienced a serious embolic event, assessed as related to treatment
- 16 out of 74 CTCL patients (21.6%) treated with vorinostat and 3 of the 68 belinostat patients (4.4%) experienced thrombocytopenia, assessed as related to treatment

1) Source: Safety population from belinostat clinical studies : TT-30 and CLN-6, I.V.

2) Source: Olsen et al., Phase IIb Multicenter Trial of Vorinostat ..., Journal of Clinical Oncology 2007; 25 (21), approved in CTCL, Oral

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

Hematological malignancies are critical indications to belinostat ...

Indications	Data support	Market potential	Competition	Belinostat clinical trials
PTCL	++	Small/medium	High	CLN-6 Pivotal phase II study (BELIEF) recruitment completed
MDS/AML	+	Medium	Low/medium	CLN-15 NCI #7285 (Odenike et al)
CTCL	+	Small	High	CLN-6
NHL/Hodgkin's	+	Medium	Medium/high	CLN-9
MM	+	Medium	High	CLN-5 CLN-16

... and solid tumors have an attractive market potential

Solid tumors	Data support	Market potential	Competition	Belinostat clinical trials
CUP	Pending	Medium	Low	CLN-17 randomized phase II, data cleaning ongoing
Ovarian	+	Medium	Moderate	CLN-8 GOG-0126T
Bladder	+	Medium	Low	CLN-8
Colorectal	(+)	Large	High	CLN-4
Sarcoma	Pending	Small	Low	CLN-14
NSCLC	Ongoing	Large	High	SPPI-1014-bel NCI (Bates, #8238)
Thymic carcinoma	Ongoing	Small	Low	NCI (Giacone, #8602 and #8174)
Heptocellular carcinoma	(+)	Medium	Moderate	NCI (Yeo, #7237)

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

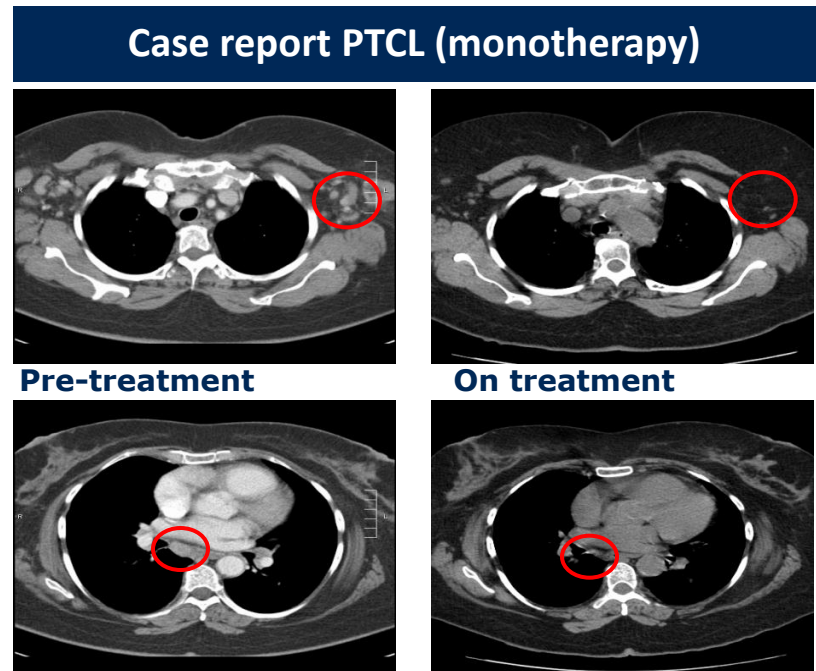
PTCL characteristics

- 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



Pivotal PTCL study BELIEF successfully enrolled

FDA Interactions

Special Protocol Assessment (SPA)

- Pivotal trial BELIEF in place with an ORR of at least 20%

Orphan drug

- Designation granted

Fast track

- Designation granted

NDA submission

- Planned for 2012

Study facts

Protocol design

- Open-labelled, multi-center, prospective, phase IIb pivotal trial
- Enrollment of 129 patients completed
- Dosing
 - I.V., 1000mg/m², days 1-5 every three weeks

DMC outcome

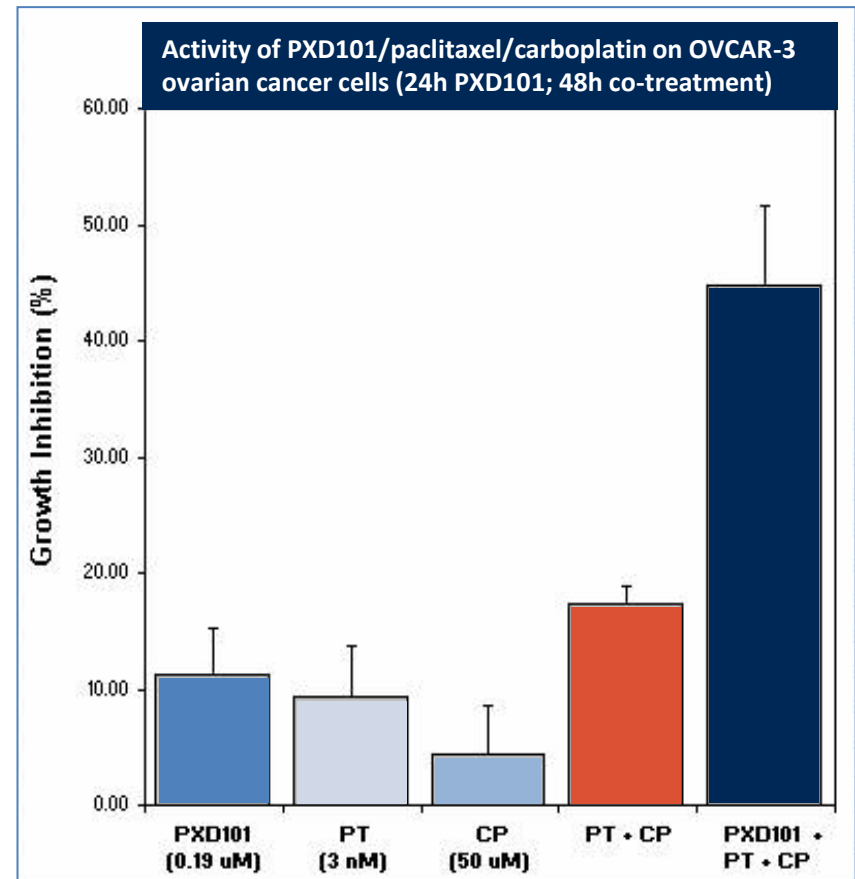
- Positive interim analysis in March 2011
 - Safety and futility – based on <5 responses in 45 evaluable patients

BELIEF

- **BEL**inostat In patients with relapsed or rEFractory Peripheral T-Cell Lymphoma

Solid tumor pre-clinical rationale: Synergistic effect in combination with chemotherapy

- Pre-clinical work demonstrated good synergies between belinostat and standard of care anti-cancer drugs
- Carboplatin and paclitaxel are the backbone in the treatment of many malignancies, i.e.:
 - CUP 1st line
 - NSCLC 1st line
 - Ovarian cancer 2nd line
 - Bladder cancer 2nd line



CUP study may support PoC of the BelCaP triple combination in the treatment of solid tumors

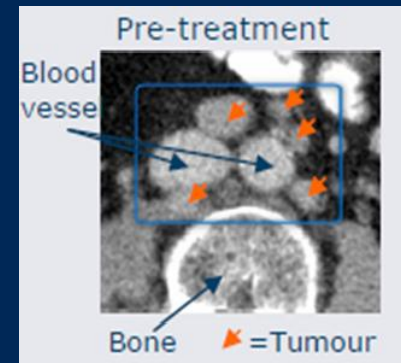
- Randomized, controlled phase II study enrollment of 89 patients completed
- Data cleaning ongoing
- Primary endpoint PFS:
 - Expected to be mature by H1 2012
- Secondary endpoints:
 - OS, ORR, time to response, duration of response, time to progression, and safety
- Very high hurdle rate with a PFS improvement of 60%
- Based on the modest powering and design of the CUP study and it is not expected to serve as a registration study

Promising results with BelCaP in bladder cancer phase II study*

Study outcome

- CLN-8 phase II cohort expansion part included 15 (14 evaluable) heavily pre-treated patients
- Encouraging response rates:
 - 1 CR (7%)
 - 3 PR (27%)
 - 10 SD (71%)
- Overall disease control rate (CR+PR+SD): 93%
- Median PFS 136 days (123 – NA)
- Safety profile similar to that expected of carboplatin/paclitaxel alone

Case story



Promising results with BelCaP also demonstrated in ovarian cancer

Study	Phase	Treatment	N	Status
CLN-8	I/II	Belinostat + carboplatin + paclitaxel (BelCaP) [21 patients were refractory to platinum]	35	RR = 48% in 31 evaluable patients and 24% in platin refractory with PFS = 5.5 mo
GOG126	II	Belinostat + carboplatin (BelCar) [patients were refractory to both paclitaxel and platinum]	27	3 responses needed in stage I not reached Study did not proceed to stage II

- **GOG and CLN-8 studies had major differences:**
 - **BelCar therapy was used instead of BelCaP**
 - **Patients in the GOG trial had more refractory tumors**

Expanding into NSCLC indication using preferred BelCaP combination regimen

- Dose optimization study* using belinostat, starting at 1000 mg/m² and escalating at 200 mg/m² steps
- Total number of patients expected to be ~35
- Study objective:
 - To evaluate the maximal tolerated dose of belinostat in combination with full doses of carboplatin and paclitaxel in previously untreated patients

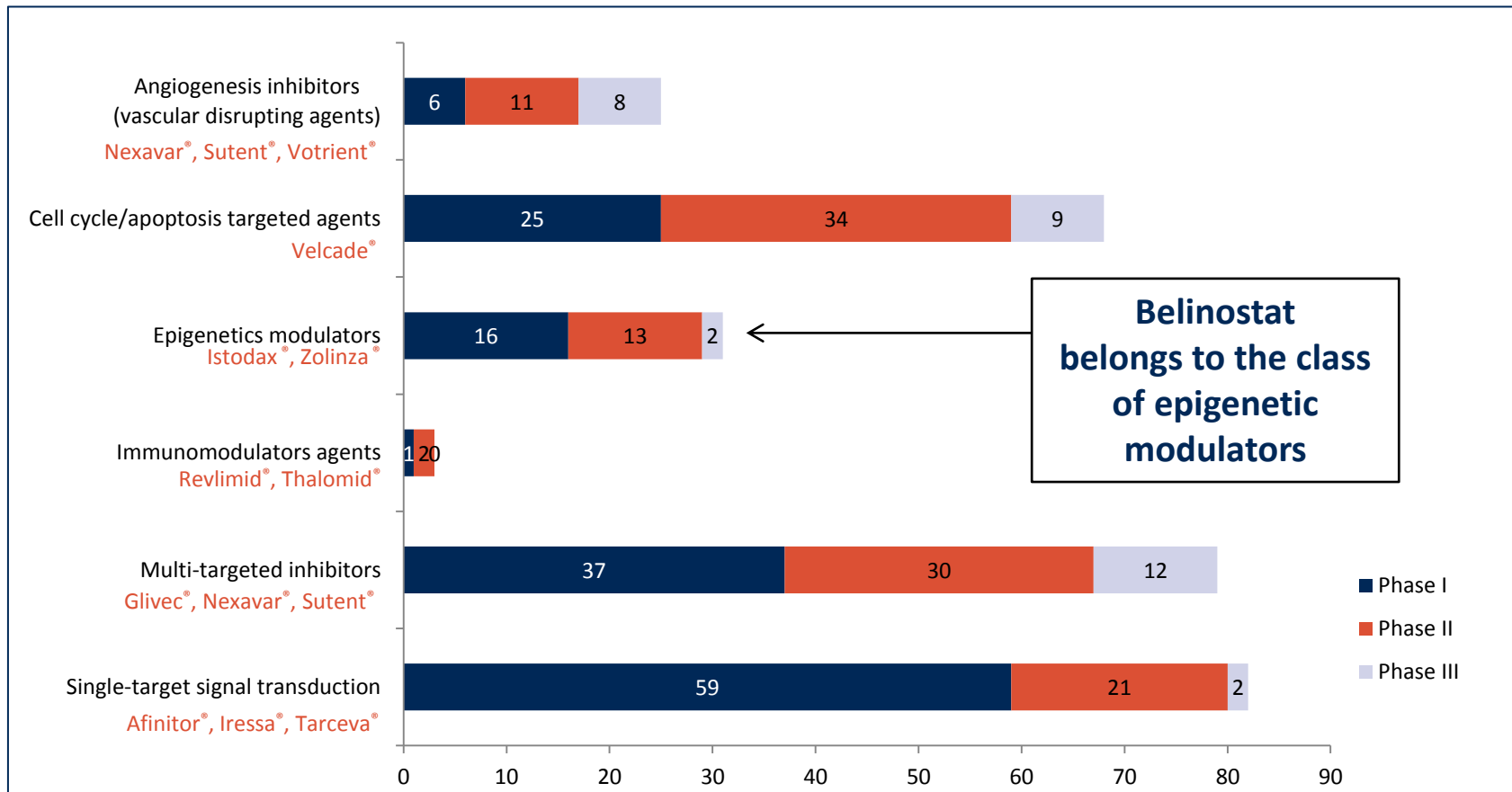


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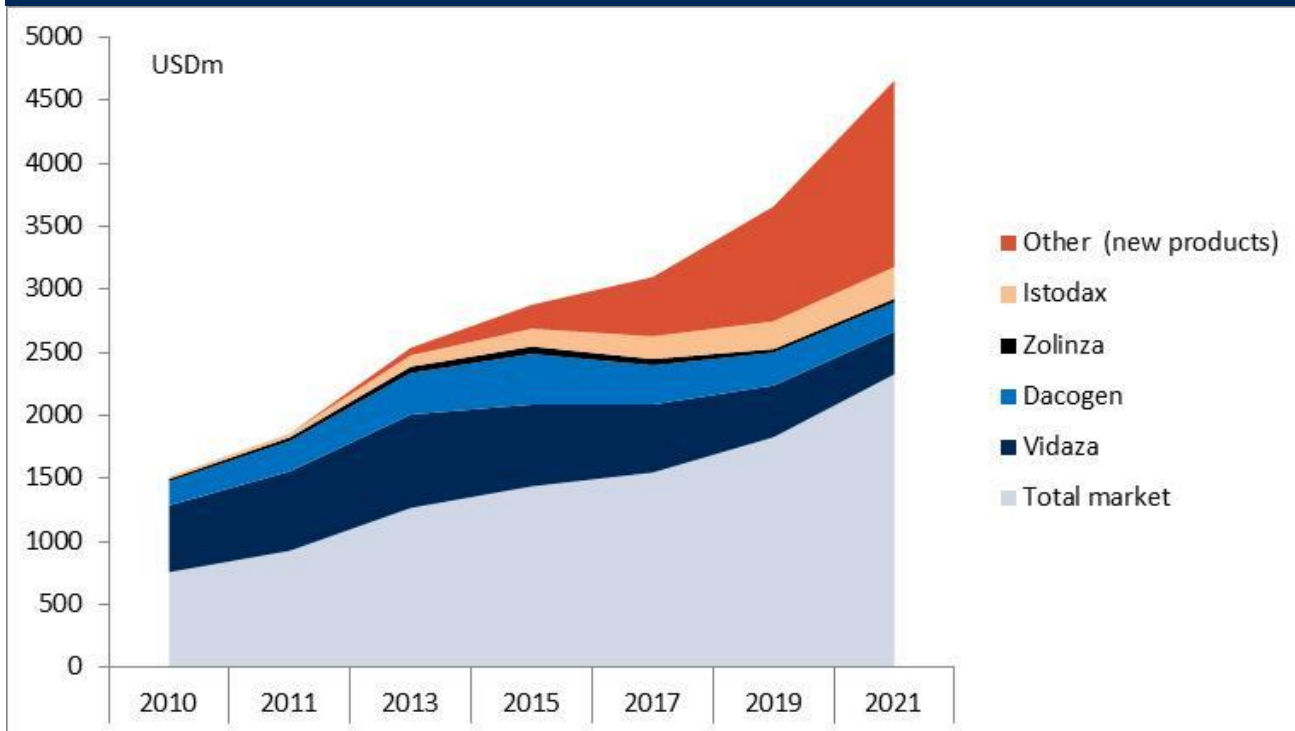
Targeted therapies clinical trials landscape

Molecular targeted cancer therapies – small molecules and marketed products



Epigenetics therapy market is estimated to triple up to USD 2.3bn in 10 years

Epigenetic therapies global revenue forecast 2011-2021



- New products are estimated to drive growth of the epigenetics therapeutic market
- US will represent approximately 2/3 of the market
- Patent expiry (US); Vidaza (2011), Dacogen (2013), Zolinza (2015), Istodax (2021)

The HDACi trial landscape in hematology is maturing ...

Generic name Brand name Company	Belinostat n/a Topotarget	Vorinostat Zolinza® MSD	Romidepsin Istodax® Celgene	Panobinostat Faridak® Novartis	Resminostat n/a 4SC
PTCL	II pivotal	II	On market	II	
MDS	I/II	II		II	
AML	I/II	II	II	II	
CTCL	I/II	On market	II	I	
Hodgkin's	I	II		II	II
MM	II	III	II	III	

... while greater opportunities exist in the solid tumor area

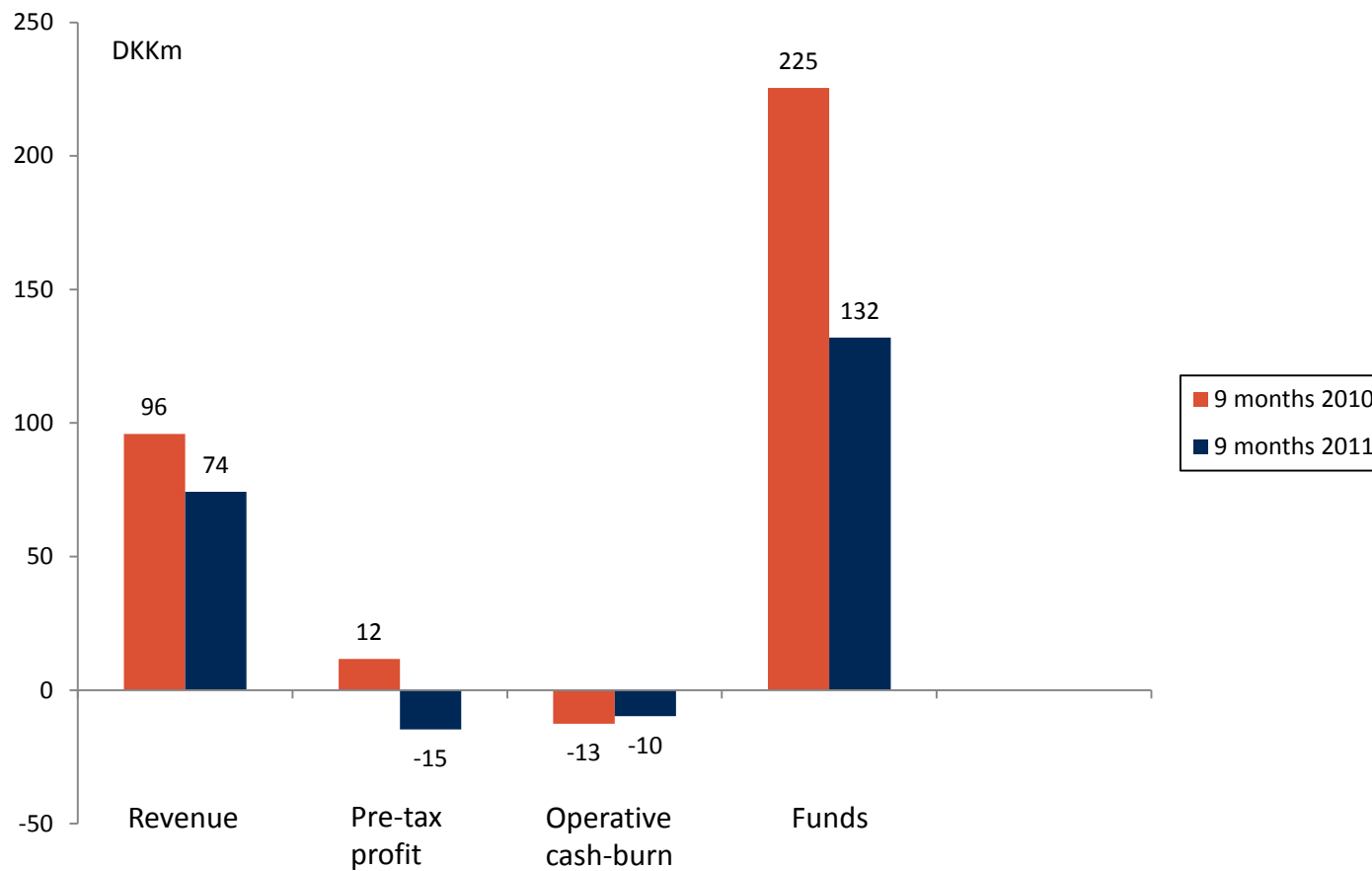
Generic name Brand name Company	Belinostat n/a Topotarget	Vorinostat Zolinza® MSD	Romidepsin Istodax® Celgene	Panobinostat Faridak® Novartis	Resminostat n/a 4SC
Bladder	II				
Ovarian	II	II			
CUP	II				
Heptocellular cancer	II	I		I	II
Sarcoma	I/II			I/II	
NSCLC	I/II	II/III	I/II	II	
Thymic carcinoma	II		II	II	
Colorectal	Ib	II	II	I	II

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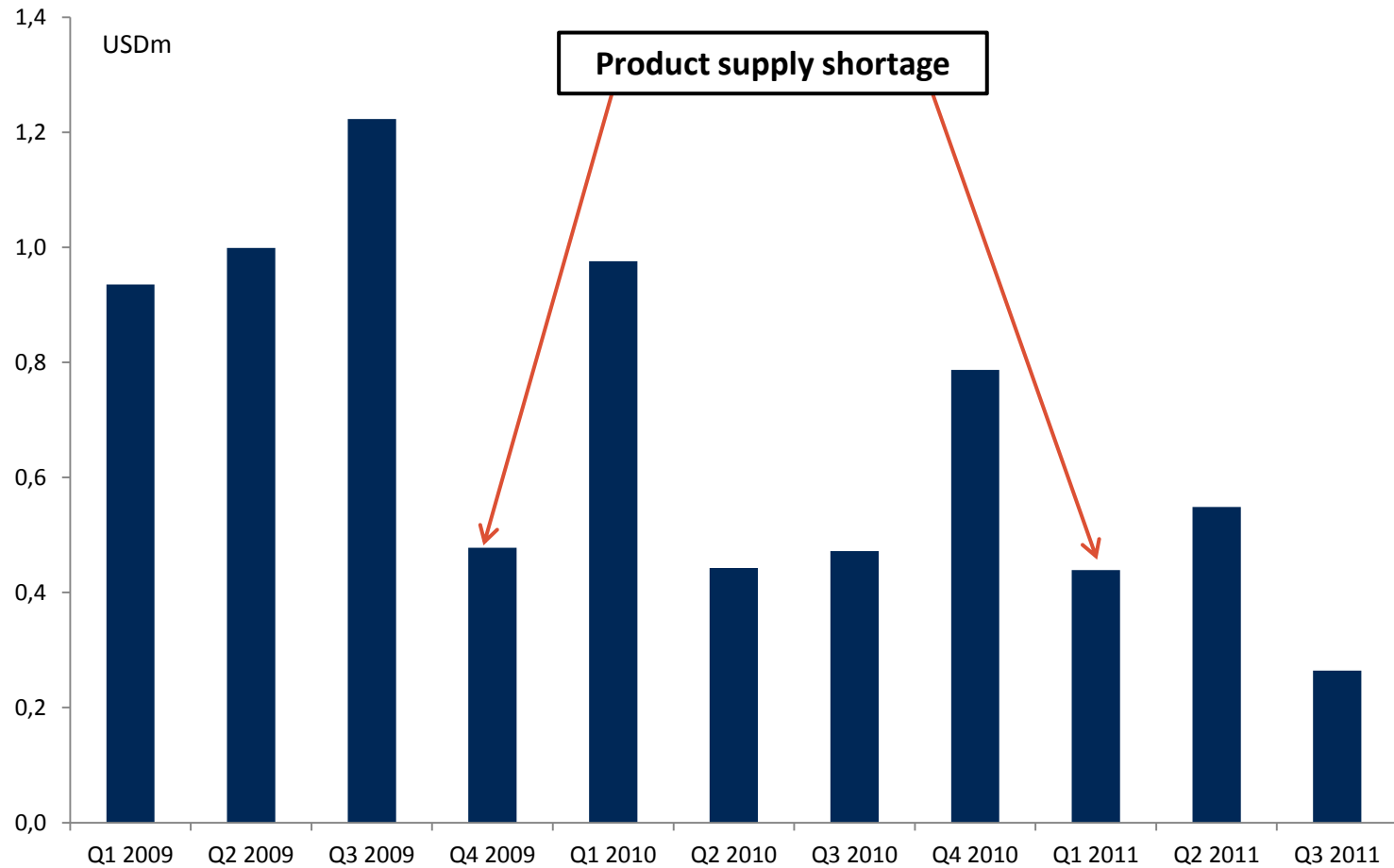
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Financial runway takes us into 2013

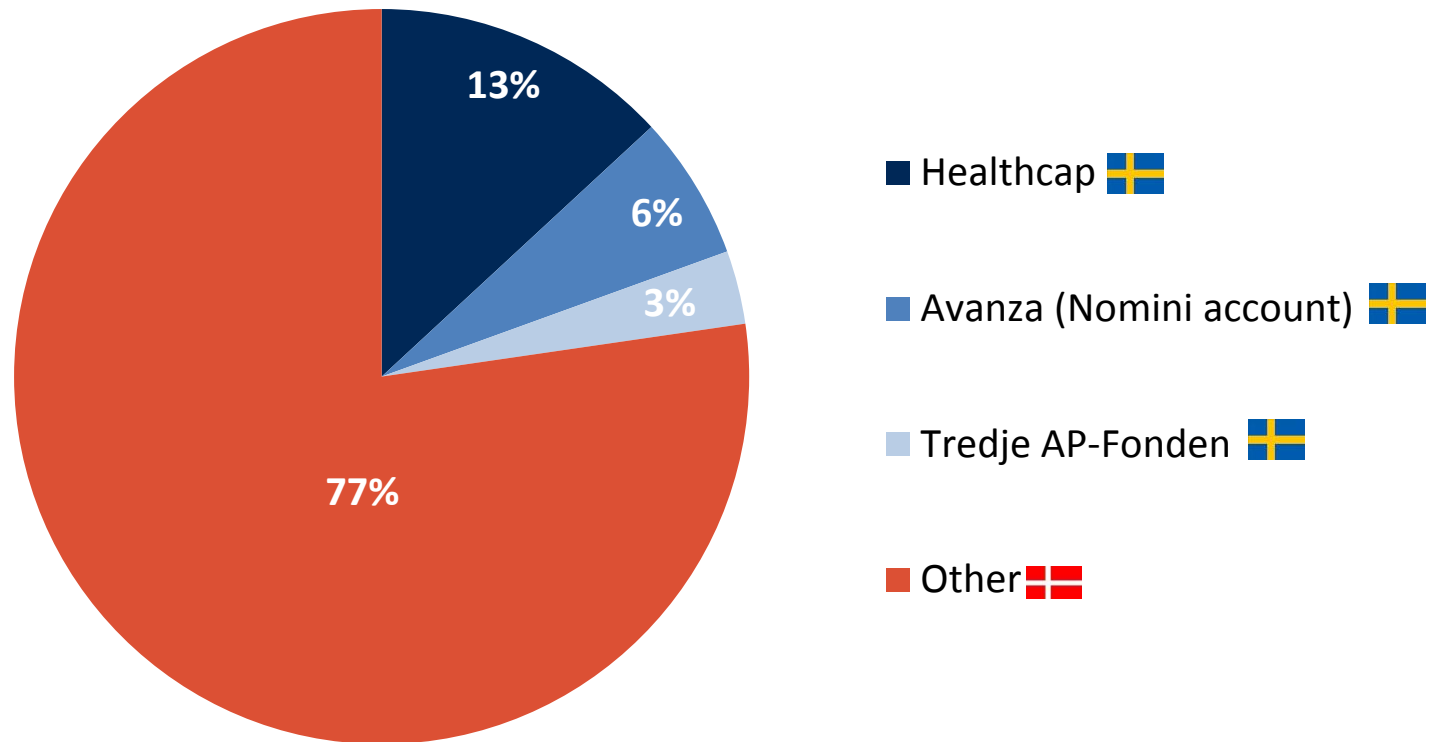
Key financial figures September 31, 2011



Challenges still prevail in Totect® sales

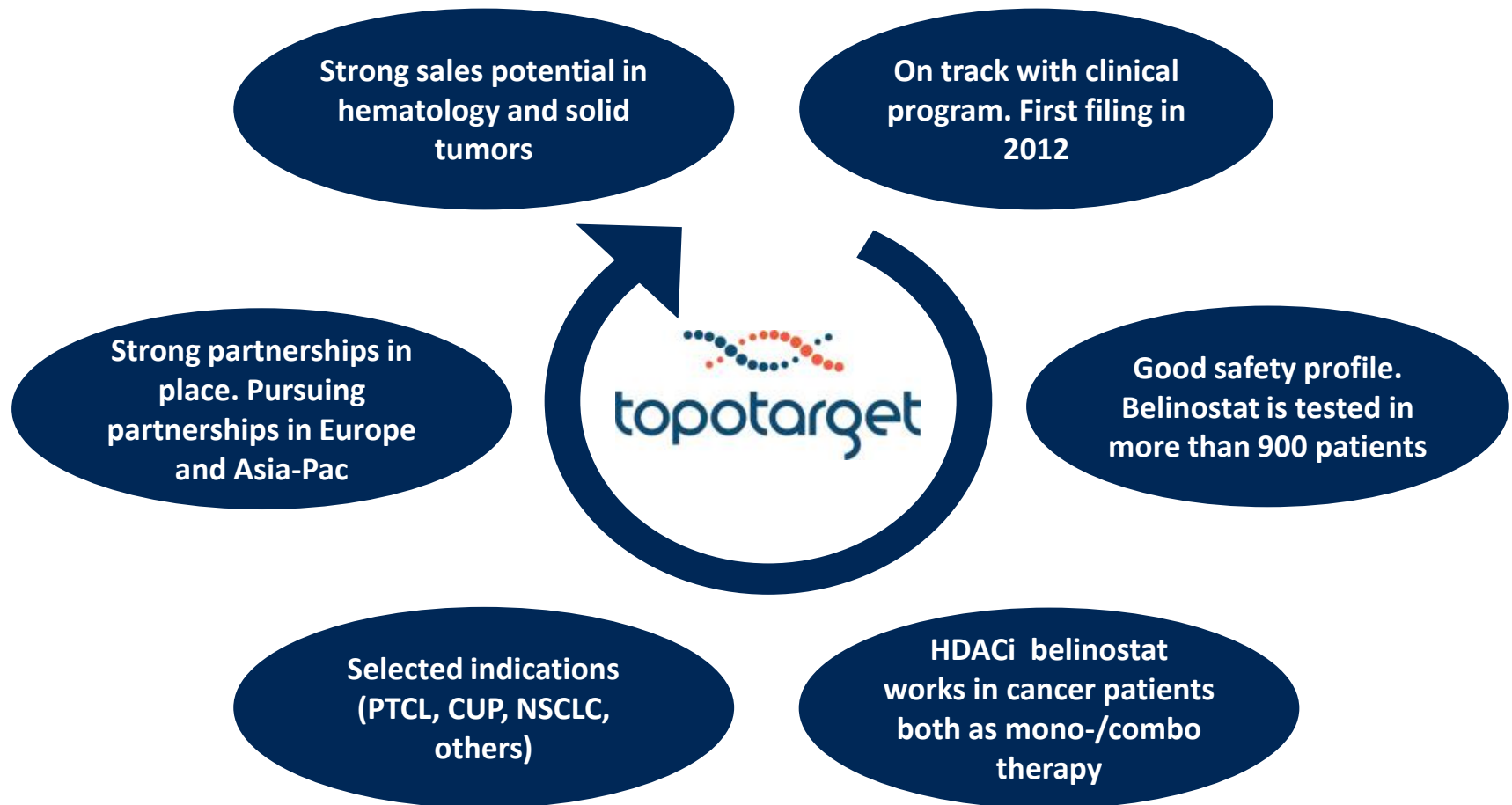


The goal is to increase the share of institutional investors

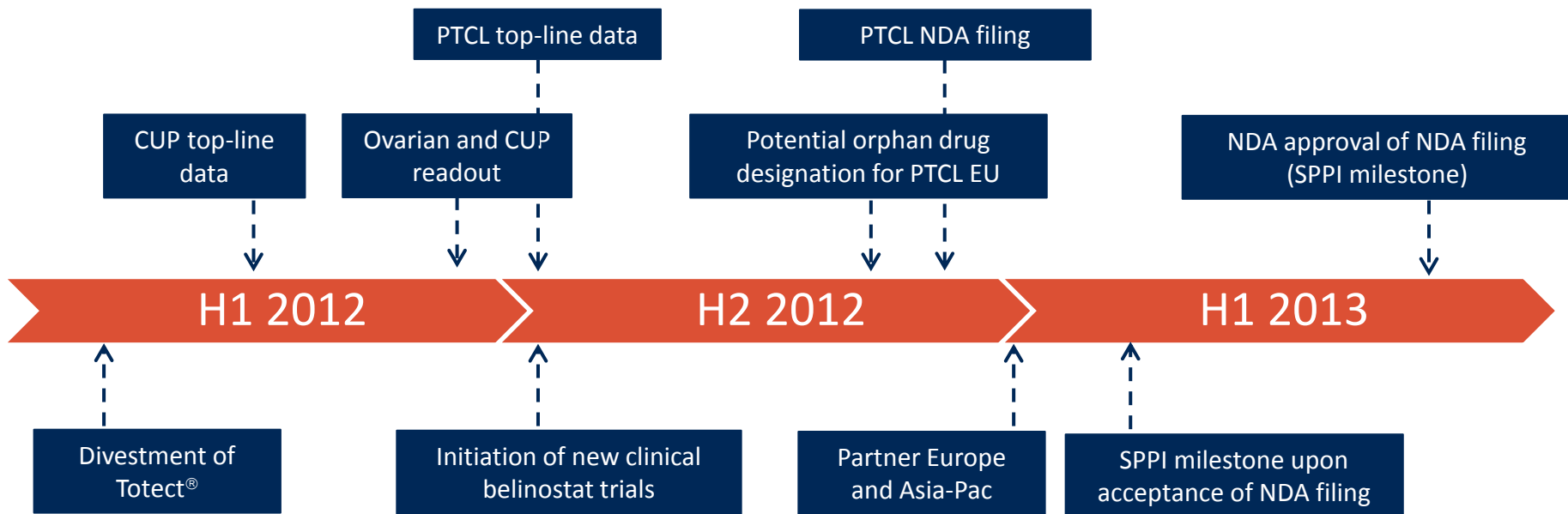


Shareholder structure based on voting rights as of October 1, 2011

Summary – building up a strong commercial profile for belinostat



Triggers* expected to increase the value – a data-driven process



*) Timelines are illustrative only

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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. The background is a blurred laboratory setting. The text "Thank you! Q&A" is centered in the middle panel.

Thank you!
Q&A