



Topotarget A/S

**Proinvestor Life Science seminar
Copenhagen 14 September 2011**

Safe harbour statement

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Topotarget at a glance (1)

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

- Headquartered in Copenhagen, Denmark (Medicon Valley) with ~46 employees
- Focused on development and commercialization of new innovative drugs for cancer treatment
- Belinostat: Lead drug candidate and compelling Histone DeAcetylase inhibitor (HDACi)
- Totect[®] (for anthracycline extravasation): Marketed by Topotarget USA, Inc. in the US

Listing	NASDAQ OMX Copenhagen
Symbol	TOPO.CO
Market capitalization (as of 13 September 2011)	€ 35m
No. of shares (as of 30 June 2011)	132,652,050

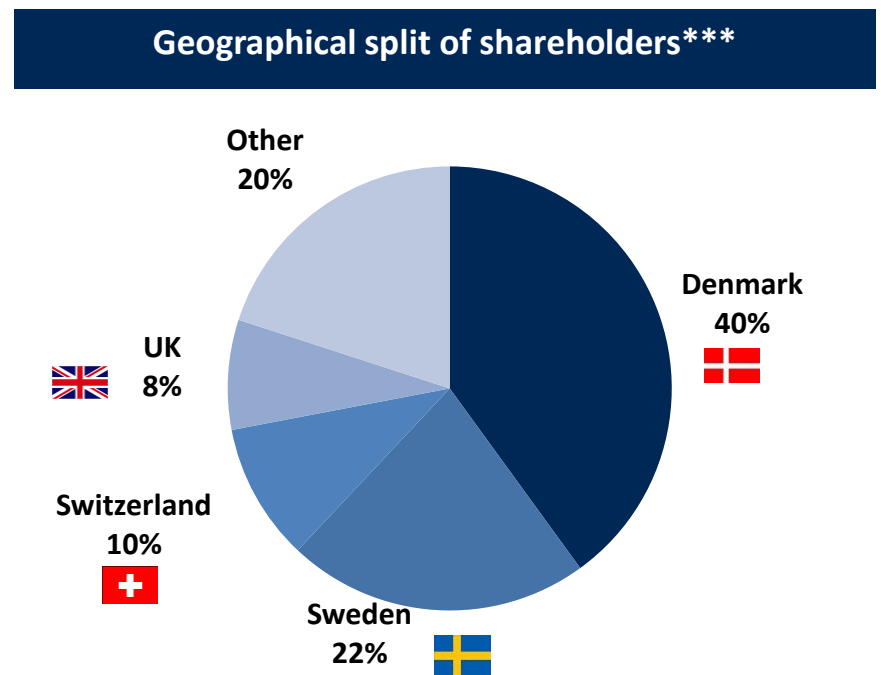
Topotarget at a glance (2)

- Topotarget has, based on current plans, sufficient cash resources until at least 2012, without taking into account potential milestones

Shareholder	Ownership
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 – making a difference to cancer patients and shareholders

Creating shareholder value

Track record

Proven track record only 7 years from idea to launch

Totect®

Pipeline

Novel cancer drug
Target:
HDACi

Partnerships

Strong partnerships

Unmet market need

Solid tumor
Hematological malignancies
Solid commercial potential

Strong belinostat profile

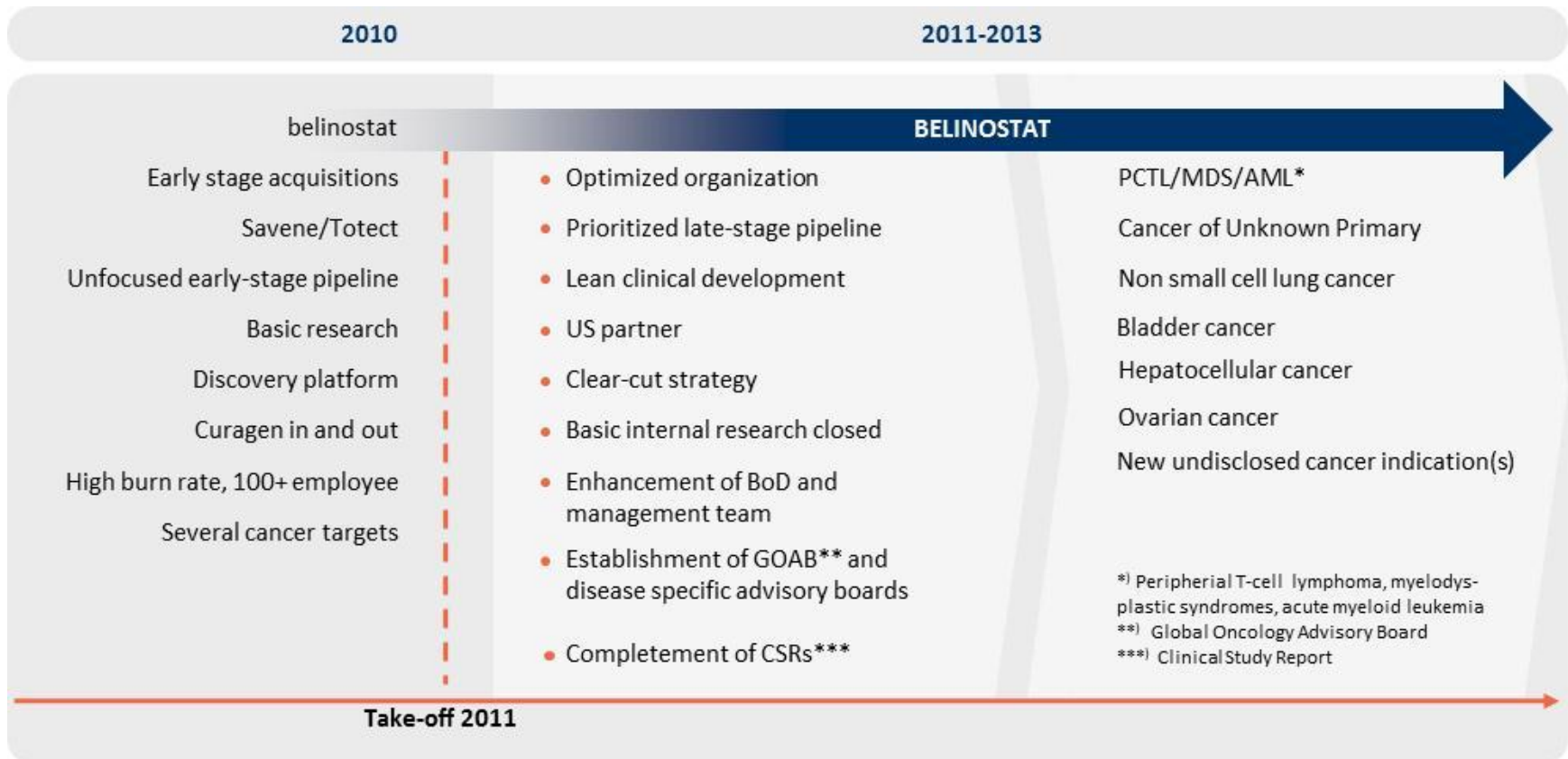
Efficacious
Robust safety
Strong IP
Large data base
Mono- and combination therapy

Belinostat partner agreement with Spectrum Pharmaceuticals, Inc.

- Agreement, 2 February 2010
- USD 30m cash upfront
- Potential value USD 320m in milestones
- + 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 right of negotiation to China



Focus on belinostat – core of new strategy



Topotarget progress on-track

PTCL

- Data Monitoring Committee gave positive recommendation to pivotal PTCL study in March 2011
- Enrollment are on track and to be concluded by H2 2011

CUP

- Collecting and cleaning data
- Release in H2 2011

CLN-9

- (Oral belinostat) completes enrolling

Scientific progress

- Belinostat abstracts published at ASCO, ESMO, ASH, among others
- Completion of GOAB (Global Oncology Advisory Board)

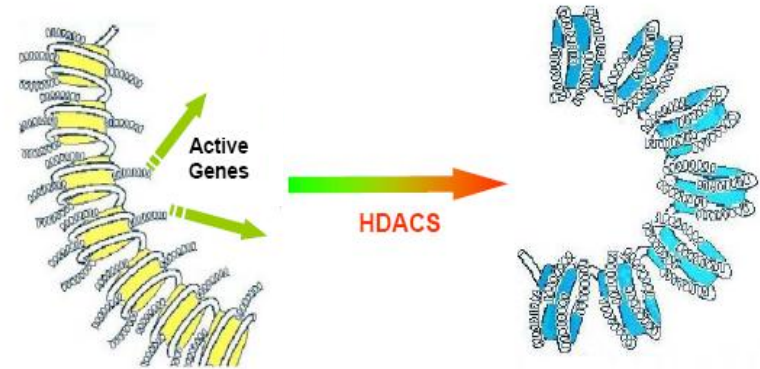
Enhancement of management

- Two additional members of the Board of Directors elected at EGM 29 August 2011; Dr. Gisela Schwab and Dr. Karsten Witt

Histone DeAcetylase inhibitors (HDACi)

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

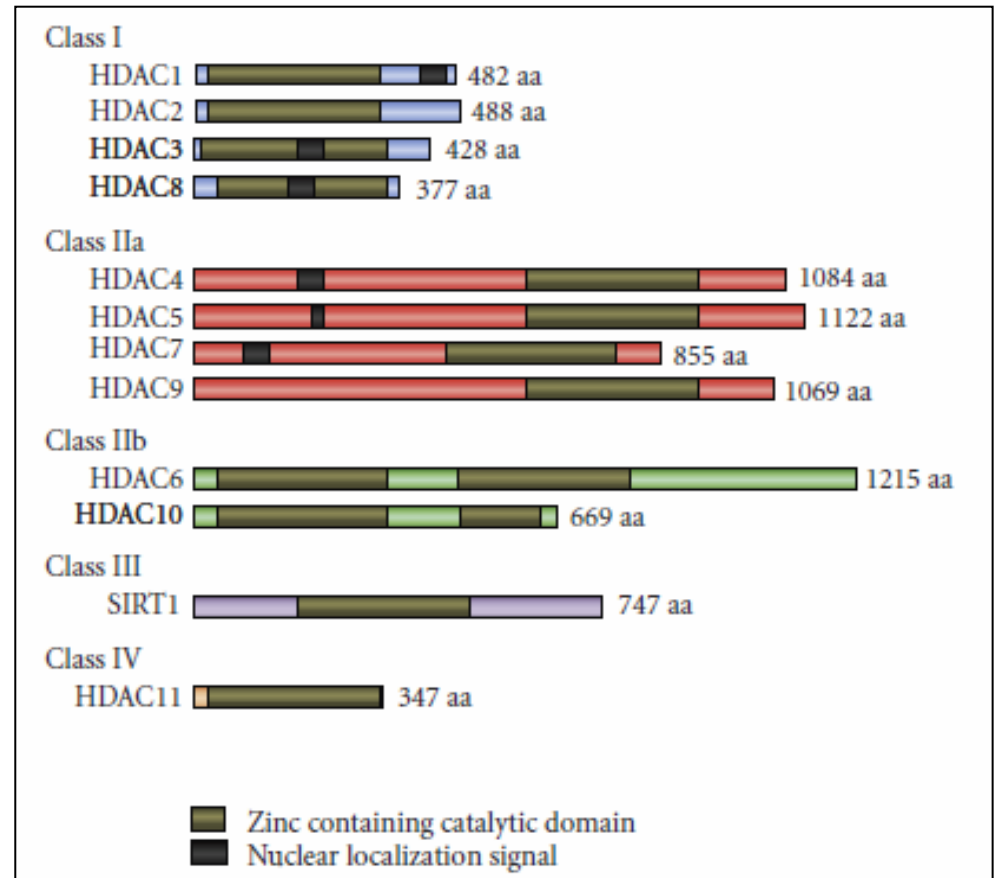
Belinostat works in both solid tumors and hematological malignancies

HDAC classification tip-offs on potency

Belinostat is a class I/II HDACi

- In order to effectively treat the tumor several HDAC members need to be modulated
- Therefore the multi-potent HDACi belinostat may be more effective than highly selective HDAC inhibitors

HDAC classes



Belinostat – compelling profile

Supercharging chemotherapy

Efficacious

- In solid and hematological malignancies
- Synergistic effect with established therapies

Flexible administration

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

Strong patent position

- Composition of matter = 2021;
IV formulation = 2026

Encouraging safety profile

- Other HDACs have significant side effects with hematological toxicity in drug combinations
- Shown to be safe in the clinical use (≈ 900 patients), with an excellent safety and cardiac tox profile and minimal bone marrow toxicity

Ability to combine

- In highest dose combined with main established chemotherapies and by that maximizing the commercial potential

Solid rationale for use of belinostat

Cancer is divided into two major groups:

- Hematological malignancies (10%)
- Solid tumors (90%)

It is known that a number of drugs are quite effective as single agents in hematological malignancies

- Topotarget and NCI explored several hematological malignancies using belinostat as single agent, for example: ALL, AML, MM, MDS, PTCL, CTCL
- Most of these studies were phase I or I/II
- Key clinical trial within hematological malignancies is the pivotal BELIEF study in PTCL

It is well-established that in solid tumors, most drugs work as combination treatment only

- Belinostat, Carboplatin and Paclitaxel are used as the back bone of combinations therapies in our clinical studies within solid tumors

Strong rationale for using belinostat is endorsed by the strong academic interest and the many investigator trials (more than 15)

Potential belinostat new indications

Hematological malignancies (i.v.) – first entrance to market

Hematological malignancies	Data support	Market potential	Competition	Belinostat clinical trials
PTCL	++	Small	High	CLN-6, pivotal study (BELIEF) is ongoing
MDS/AML	+	Medium	Low/medium	CLN-15 NCI (Odenike, # 7285)

Potential belinostat new indications

Solid tumor (i.v.) – attractive market potential

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

Belinostat key clinical trials 2011-2012 (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	LPFV stage I in phase II Results stage I	H2 2011
			Phase II	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

PTCL – NDA filing 2012

- promising data from CLN-6 headstarted pivotal BELIEF trial

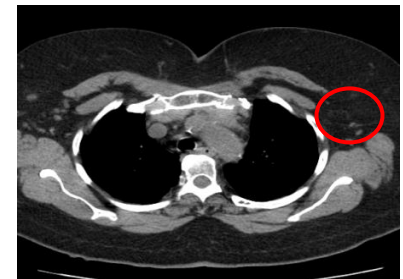
Study PXD101-CLN-6

- 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

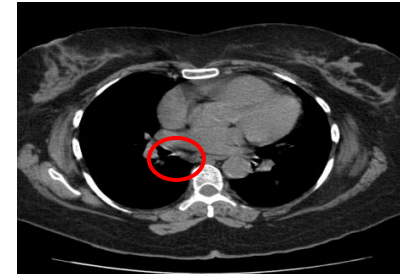
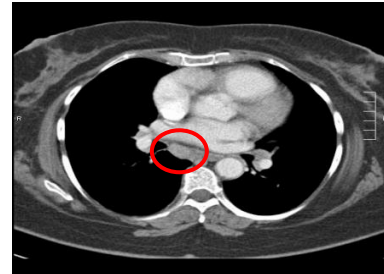
Case report PTCL (monotherapy)



Pretreatment



On treatment



PTCL – a niche area with unmet need

Key facts for PTCL

- Niche market. Belinostat WW peak sales US\$ 100m
- Unmet medical need
- 12,000 new cases per annum (US, Japan and EU top-5)
- PTCL is aggressive and treatment is imperative to quality of life and survival

PTCL definition

- Peripheral T-cell Lymphoma (PTCL) is a sub-type of non-Hodgkins lymphoma
- Aggressive, high-grade cancer
- It generally has a poor prognosis
- 15% of non-Hodgkins lymphoma patients have a PTCL subtype
- Average age of patients with PTCL is 65 years

PTCL – belinostat market entrance

A multicenter, open-label trial of **BEL**inostat In patients with relapsed or **rE**fractory Peripheral T-Cell Lymphoma

- BELIEF trial is our pivotal trial in PTCL
- Target accrual 120 (to get 100 evaluable) patients
- Dosing
 - I.V., 1000mg/m², days 1-5 every three weeks
- Interim analysis (25 March 2011):
 - Safety and futility - based on < 5 responses in 41 evaluable patients
- Final data:
 - Positive outcome response rate at least 20%
- NDA submission is targeted for 2012
- Study is sponsored by Spectrum Pharmaceuticals, Inc.

Pivotal BELIEF

- regulatory status in focus

FDA interaction

- **Special Protocol Assessment (SPA)** for pivotal trial BELIEF is in place with a required response rate of 20%
- **Orphan Drug Designation**
- **Fast Track Designation**
- **NDA submission** is planned for for 2012

Recommendations from DMC

- **Interim safety assessment** and futility analysis performed after the first 45 patients with at least 1 dose of belinostat
- **No significant safety** concerns
- DMC recommends that the study **continues** according to the protocol until 100 evaluable patients are enrolled

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)
Operating profit/(loss)	822	(17.493)	2.191	7.318	(168.446)
Financial income and expenses	5.296	1.549	117	324	68.772
Profit/(loss) before tax	6.118	(15.944)	2.308	7.642	(99.674)
Tax on profit/(loss) for the period		-		-	43.985
Net profit/(loss) for the period	6.118	(15.944)	2.308	7.642	(55.689)
Other comprehensive income	-	-	-	-	-
Total comprehensive income for the period	6.118	(15.944)	2.308	7.642	(55.689)
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)
Average number of employees			46	52	50

Topotarget US Inc.

- Revenues from Totect[®] sales were DKK 5.2 million in H1 2011 (DKK 8.0 million in the same period 2010 and DKK2.4m in Q1 2011)
- Sales of Totect[®] 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[®]
- The new batch of Totect[®] 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
Assets			
Intangible assets	235.340	426.448	235.717
Property, plant and equipment	4.341	4.709	5.991
Non-current investments	937	940	972
Non-current assets	240.618	432.097	242.680
Inventories	2.324	3.051	1.625
Receivables	19.299	21.116	16.451
Cash and cash equivalents	151.727	262.113	205.068
Current assets	173.350	286.280	223.144
Assets	413.968	718.378	465.824
Equity and liabilities	364.002	420.832	360.216
Equity	364.002	420.832	360.216
Non-current liabilities	14.116	136.523	14.116
Current liabilities	35.850	161.023	91.489
Liabilities	49.966	297.546	105.605
Equity and liabilities	413.968	718.378	465.824

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
Cash flows from operating activities before interest	(53.426)	85.322	27.575
Received and paid interest etc.	26	9.824	12.524
Cash flows from operating activities	(53.400)	95.146	40.099
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	-	-	-
Cash flows from investing activities	59	36.822	34.686
Received up-front payment belinostat	-	-	-
Proceeds from the issuance of shares	-	-	138
Cash flows from financing activities	-	-	138
Increase/decrease in cash and cash equivalents	(53.341)	131.968	74.923
Cash and cash equivalents as per 1 January 2011	205.068	130.145	130.145
Cash and cash equivalents as per 30 June 2011	151.727	262.113	205.068
Cash and cash equivalents comprise:			
Deposit on demand and cash	151.727	262.113	205.068
Special-term deposit	-	-	-
Total	151.727	262.113	205.068

Belinostat key clinical trials 2011-2012 (Topotarget and Spectrum)

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Key messages

- Belinostat unique HDACi with both oral and IV formulation available
- > 30 clinical studies completed or ongoing, large academic attention
- > 900 patients being exposed to belinostat
- Good safety profile
- Shown good benefit in patients
- Ongoing registration trial in PTCL. NDA filing 2012
- Randomized phase II in CUP enrollment completed
- Substantial and untapped potential in expansion into new oncology indications
- Enhanced news flow in 2011/2012

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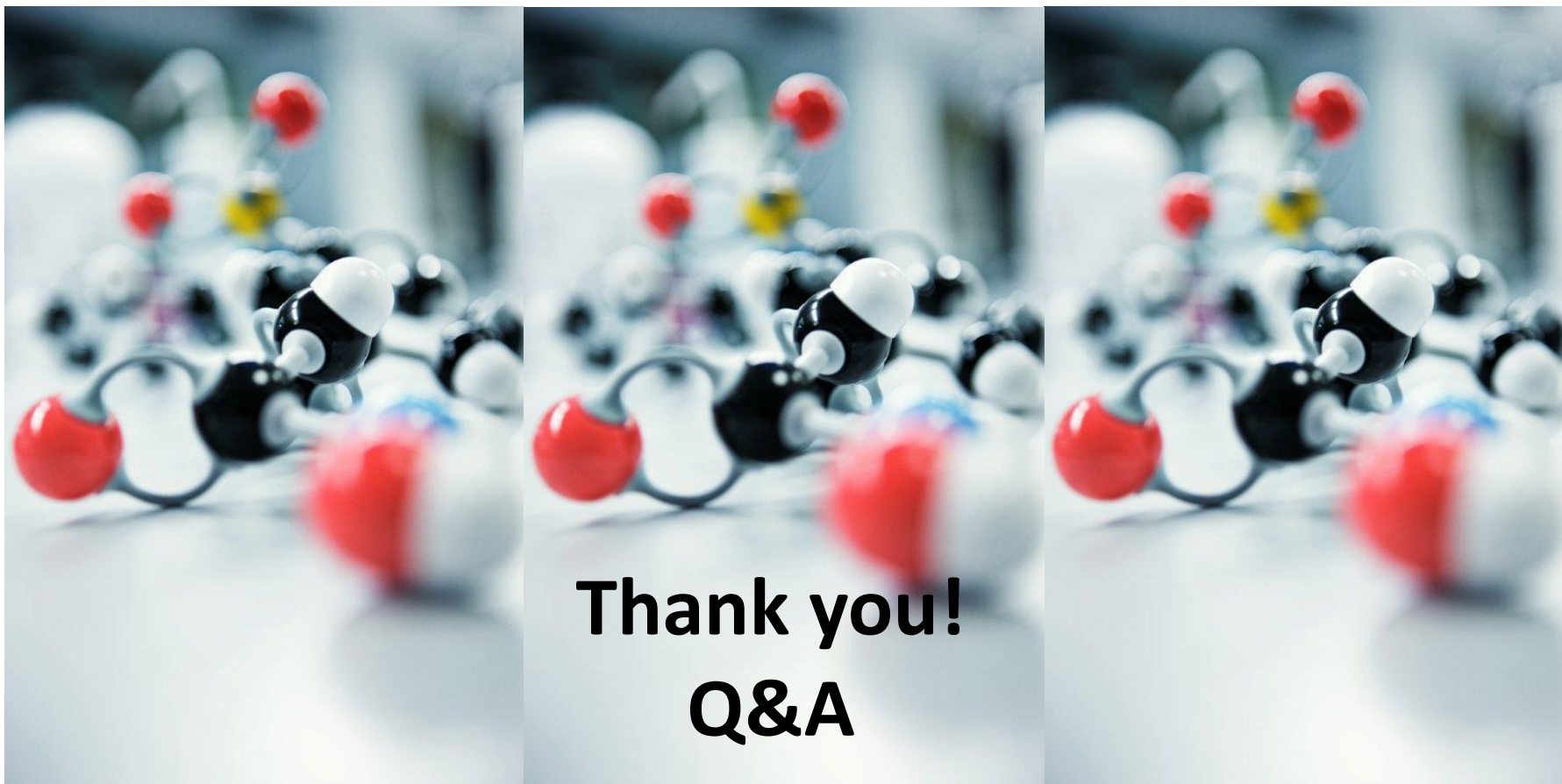
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Thank you!
Q&A