



# Topotarget A/S

Corporate Presentation

February 8, 2012

Swiss-Scandinavian Bio-Business Seminar

# 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. 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 A/S will not proceed as planned for technical, scientific, or commercial reasons or due to patient enrollment 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.

# 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 ~24 employees with a lean cost structure pursued
- Focused on development and commercialization of belinostat, a molecular targeted cancer therapy
- Divestment of Topotarget USA, Inc. and Totect<sup>®</sup> to Apricus Biosciences, Inc. closed on December 30, 2011

<b>Listing</b>	NASDAQ OMX Copenhagen
<b>Symbol</b>	TOPO.CO
<b>Market capitalization</b> (as of January 31, 2012)	€ 48m
<b>No. of shares</b> (as of January 31, 2012)	132,652,050

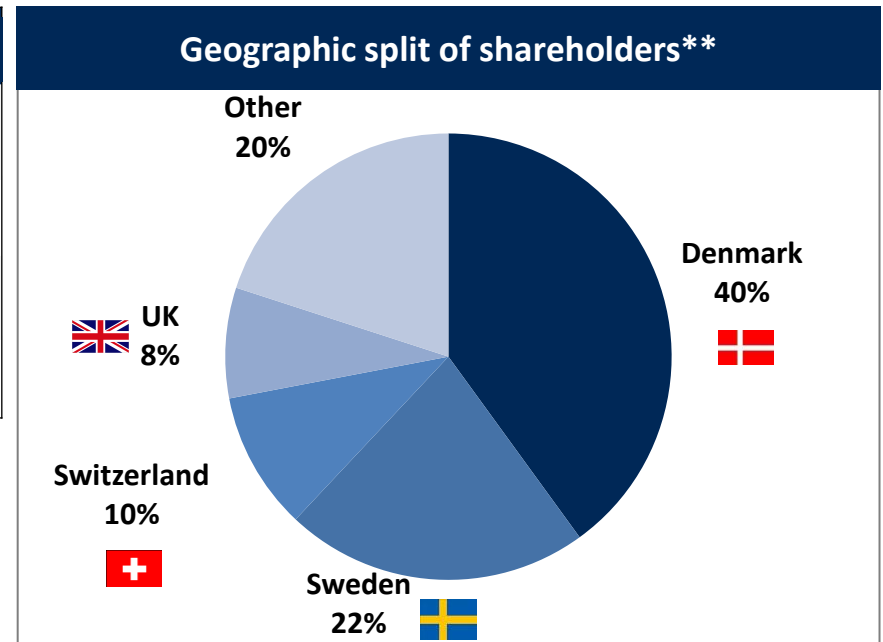
# Topotarget at a glance (2)

- 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

Shareholders**	
The 10 largest shareholders combined*	+ 30%
HealthCap funds	+ 10%
Avanza Pension	+ 5%
Tredje AP-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

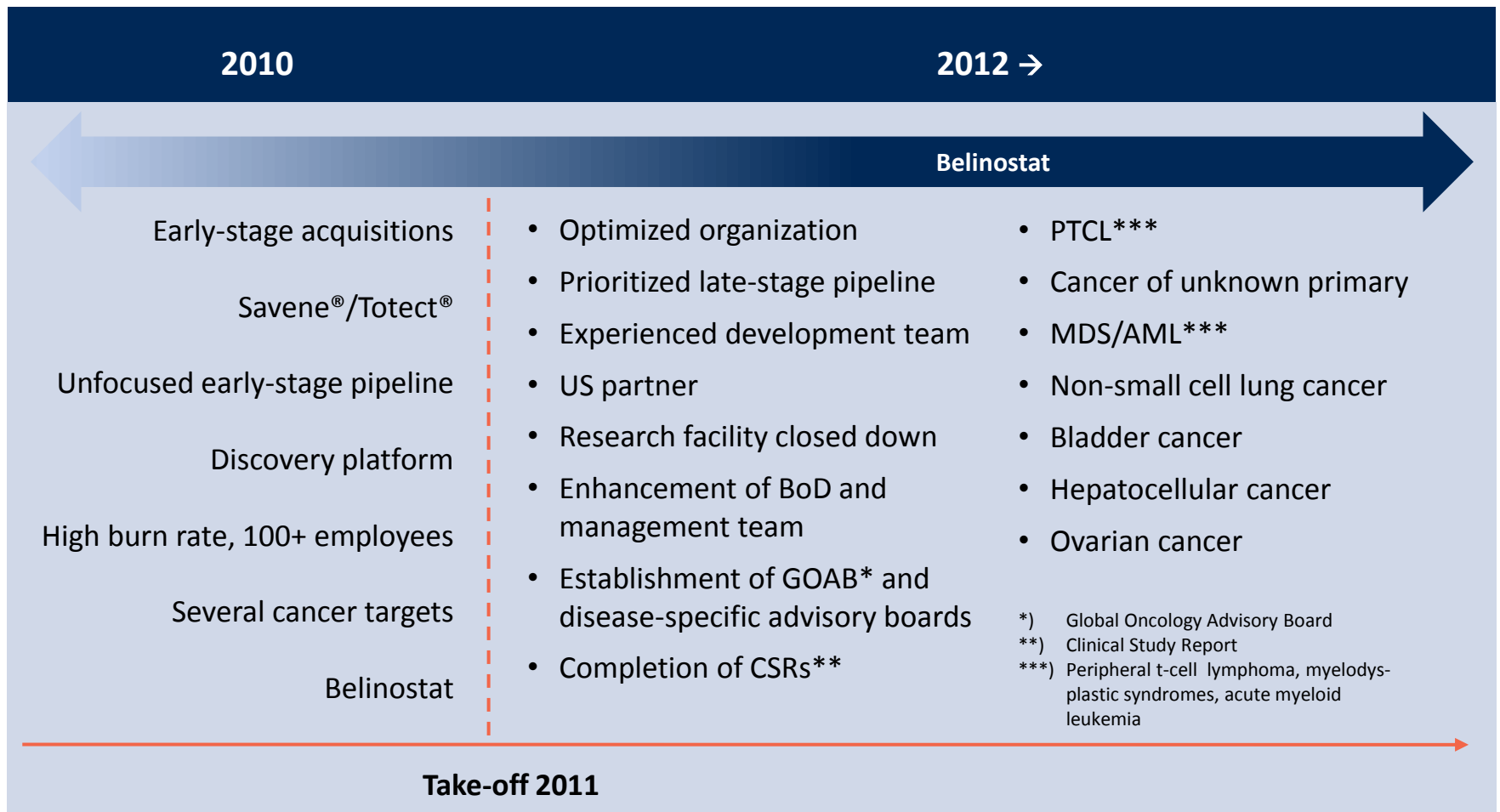
Robust safety

Strong IP

Large database

Mono- and combination therapy

# ... with a clear-cut focus on belinostat



# Our strategy is to unlock the potential of belinostat and we are on track to deliver!

## **PTCL – BELIEF study**

- Interim analysis – positive recommendation from the Data Monitoring Committee (DMC) to continue the pivotal study in March 2011
- Enrollment of 129 patients successfully concluded by September 2011. NDA submission 2012

## **CUP clinical study**

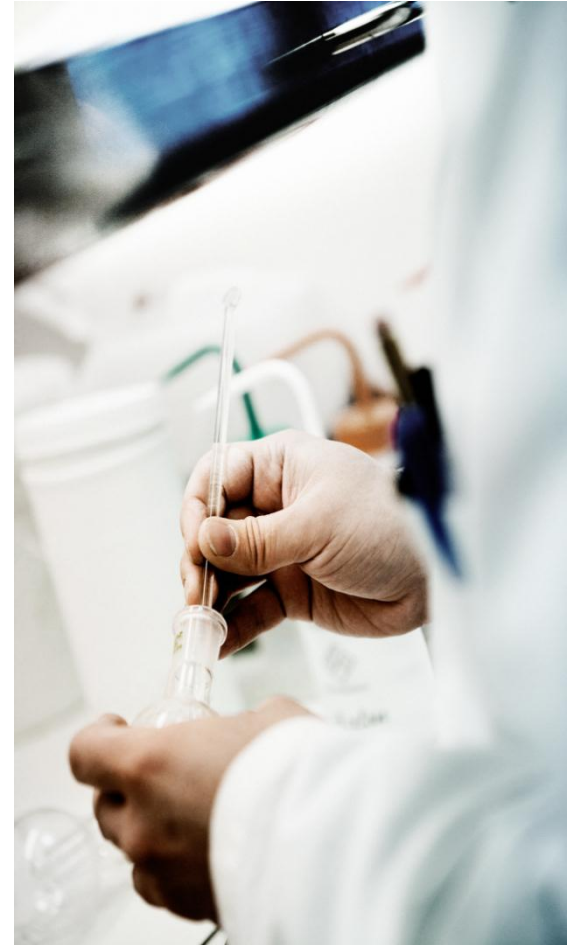
- Collecting and cleaning data
- Release of top-line data based on occurrence of PFS events is expected in H1 2012

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

- Successful enrollment of patients into oral belinostat trial

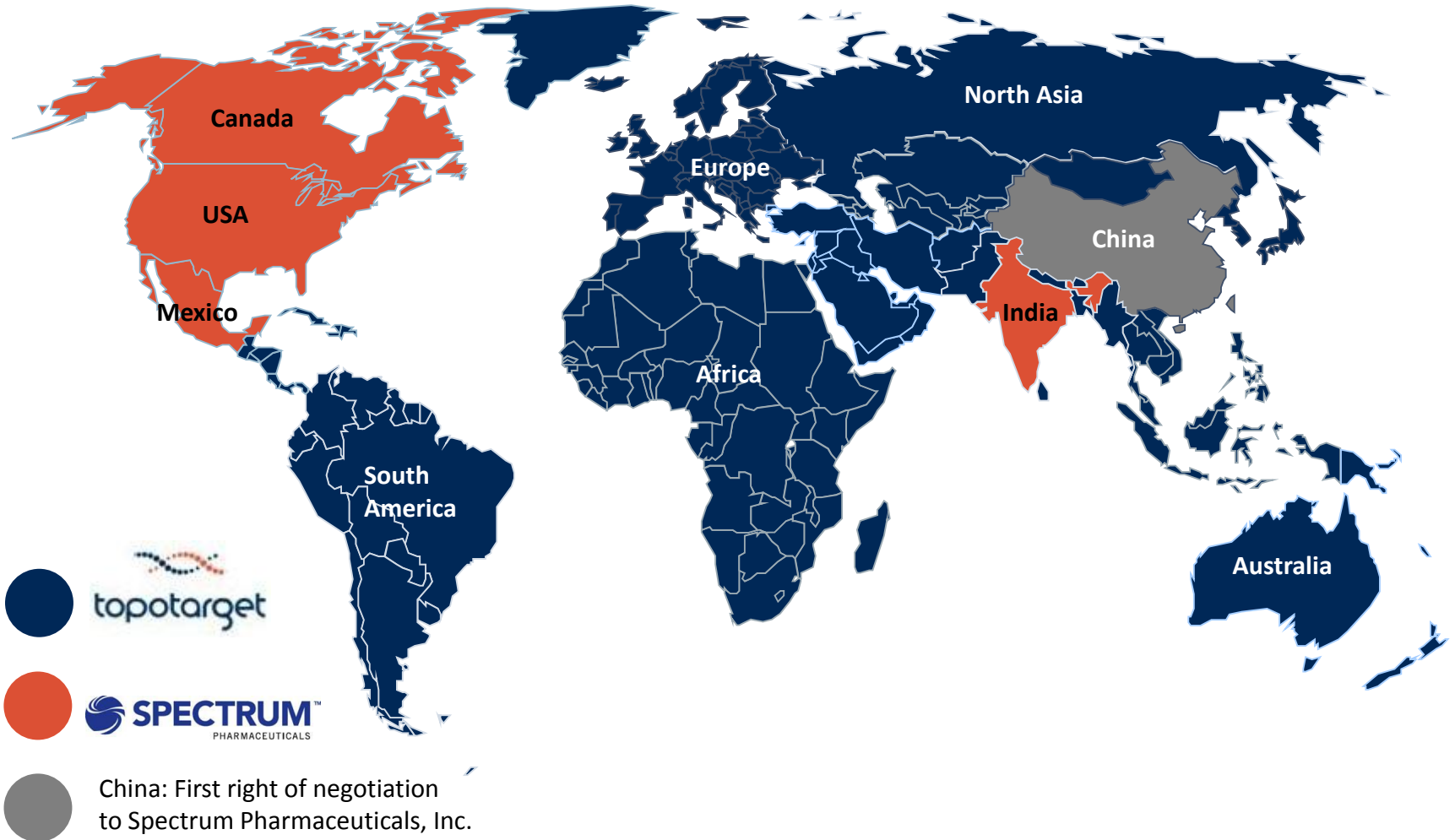
## **Scientific presentations**

- 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
- Total deal value USD 350 million, USD 30 million cash upfront, double-digit royalties
- Milestone includes 1 million SPPI shares and cash milestone at acceptance of PTCL NDA filing
- SPPI funds PTCL BELIEF trial and Topotarget funds randomized phase II CUP study
- Co-development in additional indications, cost split 70/30 (SPPI/Topotarget)
- Joint development and commercialization committees set-up
- Territory: North America and India, with a first right of negotiation to China
- SPPI is responsible for submitting an NDA on belinostat for PTCL

# Topotarget corporate presentation

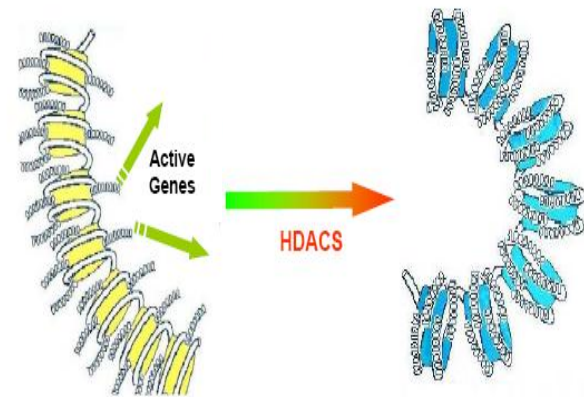
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# Histone DeAcetylase inhibitors (HDACi)

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 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 will lead to a maximized 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 ( $\approx$ 900 pts.), with an excellent safety including cardiac toxicity profile and minimal bone marrow toxicity

# 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
Solid + STS	CLN-14	TT				~55	Phase I (25) Phase II (16)	Result phase I STS expansion Recruitment complete	2012
Drug-drug	CLN-20	SPPI/TT				~39	Recruiting	n/a	n/a
NSCLC	SPI-1014 Bel	SPPI/TT				~35	Recruiting	n/a	n/a

# 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

### BELIEF

- BELinostat In patients with relapsed or rEfractory peripheral t-cell lymphoma

### Protocol design

- Open-labelled, multi-center, prospective, phase IIb pivotal trial
- Enrollment of 129 patients completed
- Dosing
  - I.V., 1000mg/m<sup>2</sup>, 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

# 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 on-going
- Primary endpoint PFS, event driven:
  - 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, it is not expected to serve as a registration study

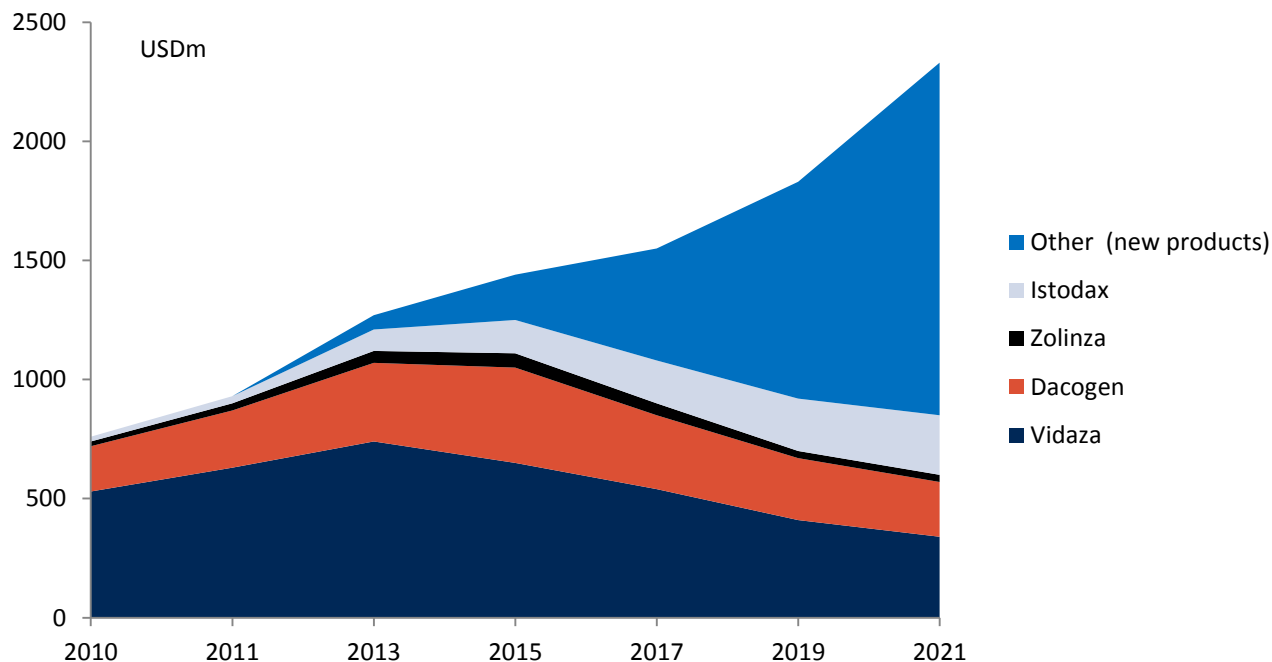


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# Epigenetics therapy market is estimated to triple up to USD 2.3bn in 10 years

## Epigenetic therapies global revenue forecast 2011-2021



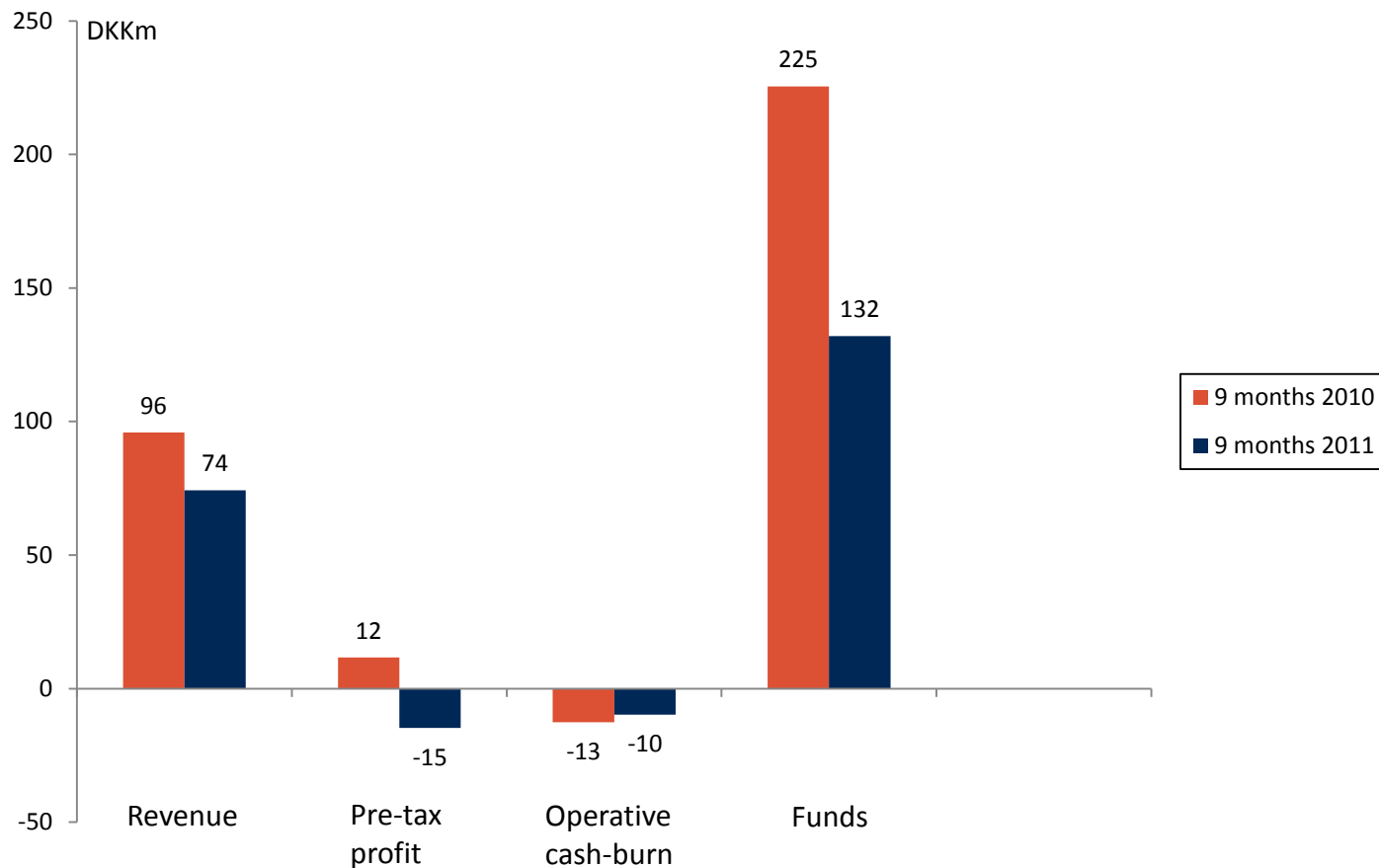
- New products are estimated to drive growth of the epigenetics therapeutic market
- US will represent approximately 2/3 of the market
- Patent expiry (US); Vidaza (2011), Dacogen (2013), Zolinza (2015), Istodax (2021)
- Belinostat patent expiry 2021 (excluding extensions)

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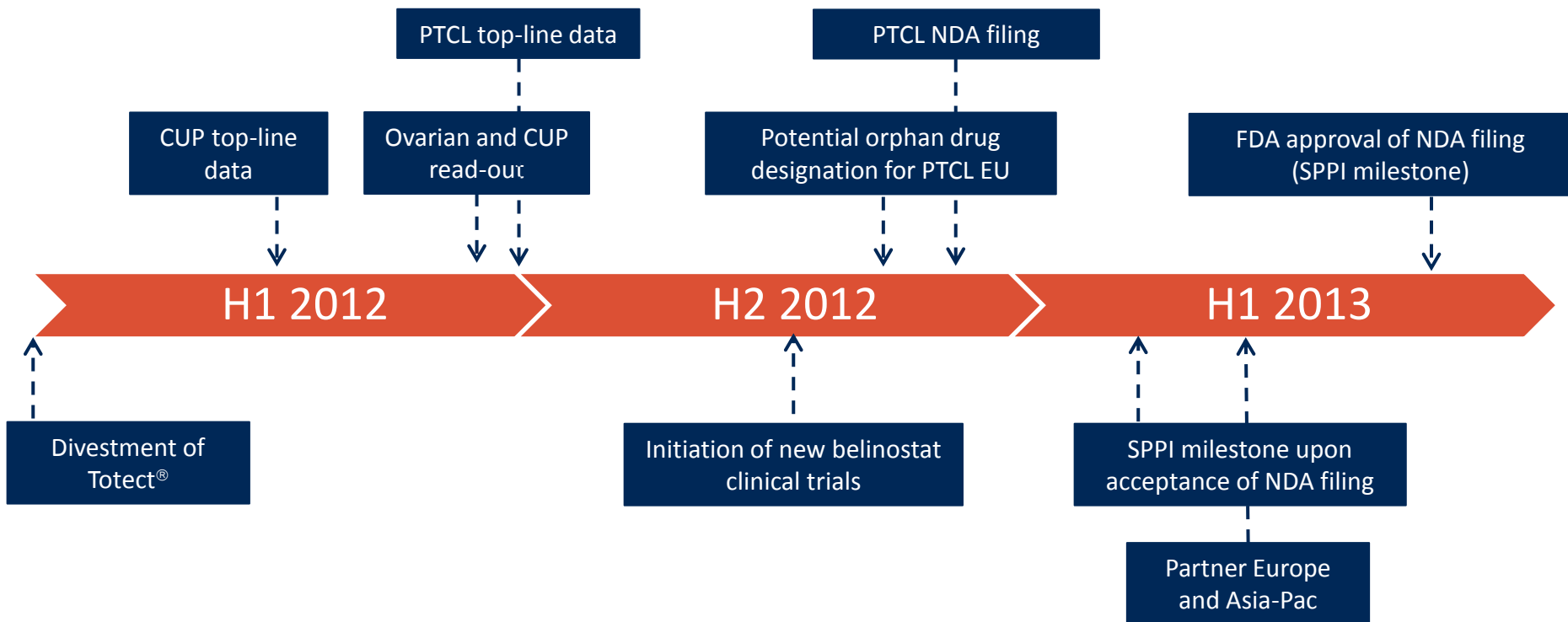
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# Financial runway takes us into 2013 excl. potential milestones

Key financial figures September 31, 2011



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



\*) Timelines are illustrative only

# Contact information

## **Francois Martelet**

*CEO*

+45 39 17 83 43 (phone)

+45 51 32 83 41 (cell)

[fma@topotarget.com](mailto:fma@topotarget.com)

## **Anders Vadsholt**

*CFO*

+45 39 17 83 45 (phone)

+45 28 98 90 55 (cell)

[afv@topotarget.com](mailto:afv@topotarget.com)

## **Topotarget A/S**

Fruebjergvej 3

DK-2100 Copenhagen



Company website:  
[www.topotarget.com](http://www.topotarget.com)



**Thank you!**  
**Q&A**