Q2 2014

targovax

Arctic Biotech Day - September 2014

Immunotherapy
- the future of cancer therapy

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Highlights

A Norwegian company founded by world pioneers in cancer immunotherapy, leveraging on product history and mature lead product TG01

Uniquely positioned with a robust product history showing promising clinical data in orphan drug indication

Large unmet medical need for cancer with RAS mutations, with no effective treatment existing today

Phase I study in Operated Pancreatic Cancer finished
- Product is safe and triggers immune responses

Phase II ongoing in internationally renowned clinical centres
- Investigating clinical effects

Successfully completed a capital increase amounting to 70 MNOK in June 2014

Clear and identified milestones going forward, with significant value inflection points during 2014 to 2016

Targovax, a Norwegian company founded by world pioneers in cancer immunotherapy

The technology was initially developed and documented by Norsk Hydro/Pronova (1990-2000) in collaboration with The Norwegian Radium Hospital

Radiumhospitalet
Comprehensive Cancer Center

Founders of Targovax are co-inventors of technology:

- **Gustav Gaudernack**

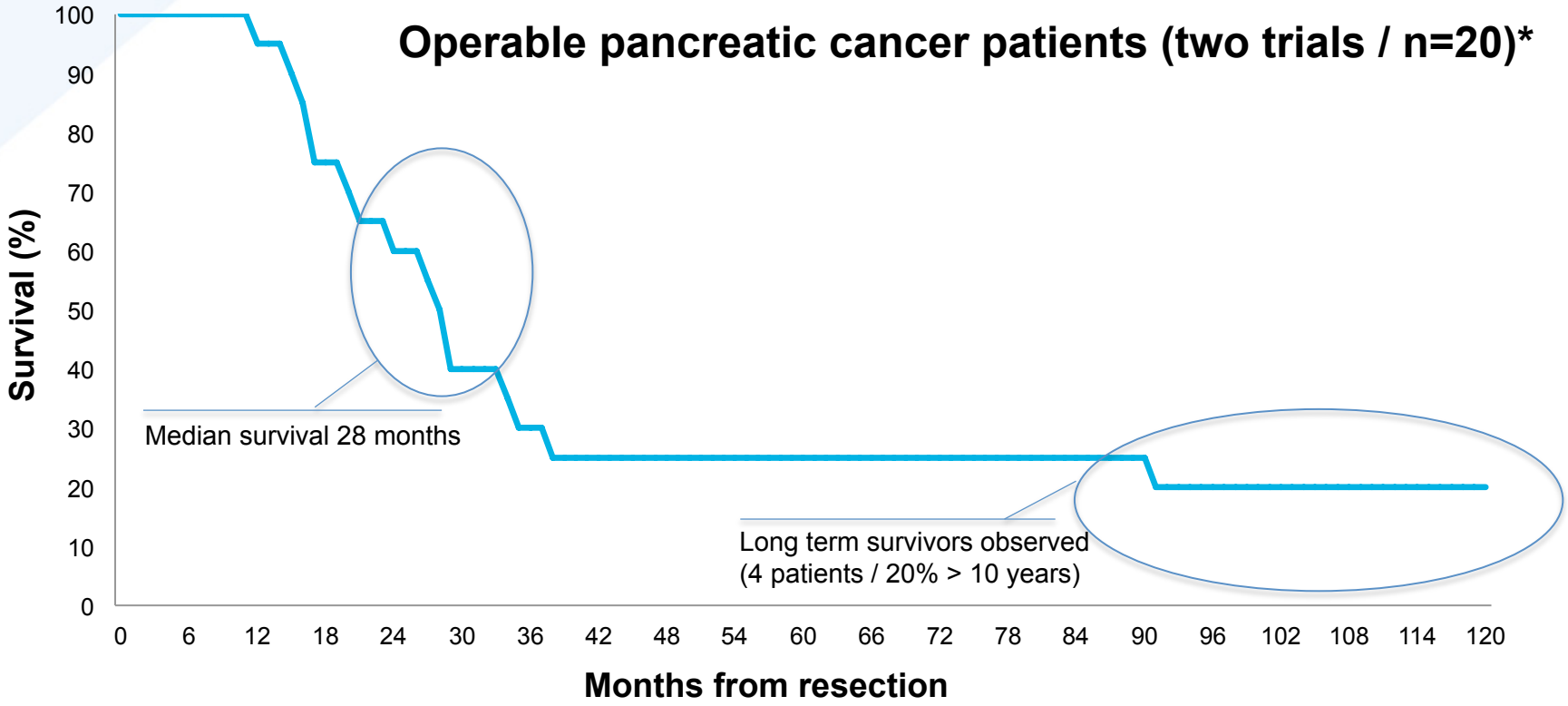
  Professor emeritus in Immunology – The Norwegian Radium Hospital
  - More than 30 years of research experience within the field of immunotherapy of cancer.
  - Internationally recognized opinion leader.
  - Author and co-author of more than 200 scientific articles and co-inventor of several patents

- **Jon Amund Eriksen**

  COO of Targovax
  - Over 30 years of experience from pharmaceutical industry; Norsk Hydro/Pronova, Pharmexa, Gemvax, Lytix, Nycomed.
  - 25 years within the field of immunotherapy of cancer

Targovax exclusively owns all established documentation

Indication of survival benefit after treatment with TG01 peptides observed in retrospective analysis



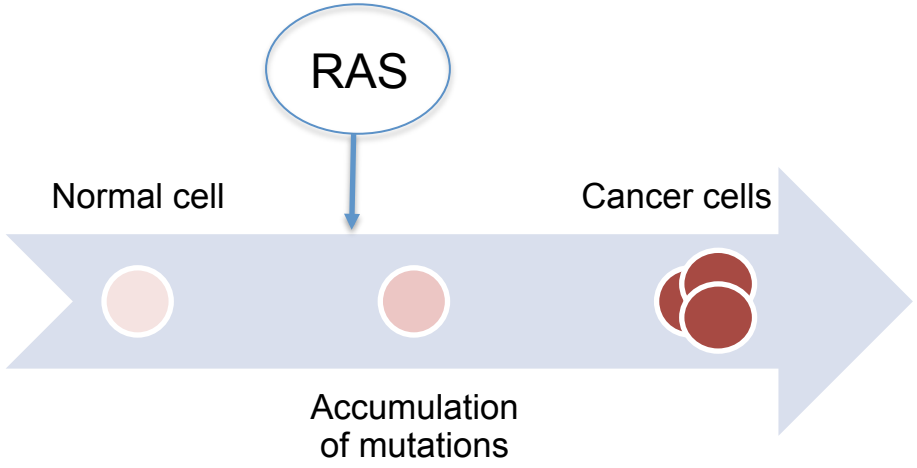
The two exploratory clinical trials with TG01 peptides performed by Norsk Hydro, 1996-2001, were designed only to investigate safety and if the patients mounted immune responses towards the RAS mutated peptides, not to assess survival. Patients were treated with one or more of the TG01 peptides. An academic, retrospective analysis of survival of patients from the two clinical trials was performed by Wedén et al as described in the paper published in 2011, many years after the clinical trials were completed. Of the 20 evaluable patients, 4 were still alive after 10 years. The observations need to be verified in clinical trials designed for investigation of survival.

*Source: Weden et al, 2011 and Clinical trial reports

RAS is a driver in cancer development

Cancer is a broad group of diseases involving unregulated cell growth.

RAS is a molecule inside the cells regulating cell division. Mutations cause sustained division, and is a driver in cancer development. RAS mutation is an early cancer marker, present in approximately 20% of all cancers*.



RAS mutations:

- ~ **90%** Pancreatic cancer
- ~ **40%** Colorectal cancer
- ~ **25%** Lung cancer
- ~ **20%** Malignant melanoma

No effective treatment exist for cancer with RAS mutations

* Prior et al, 2012

Large unmet medical need for cancer with RAS mutations

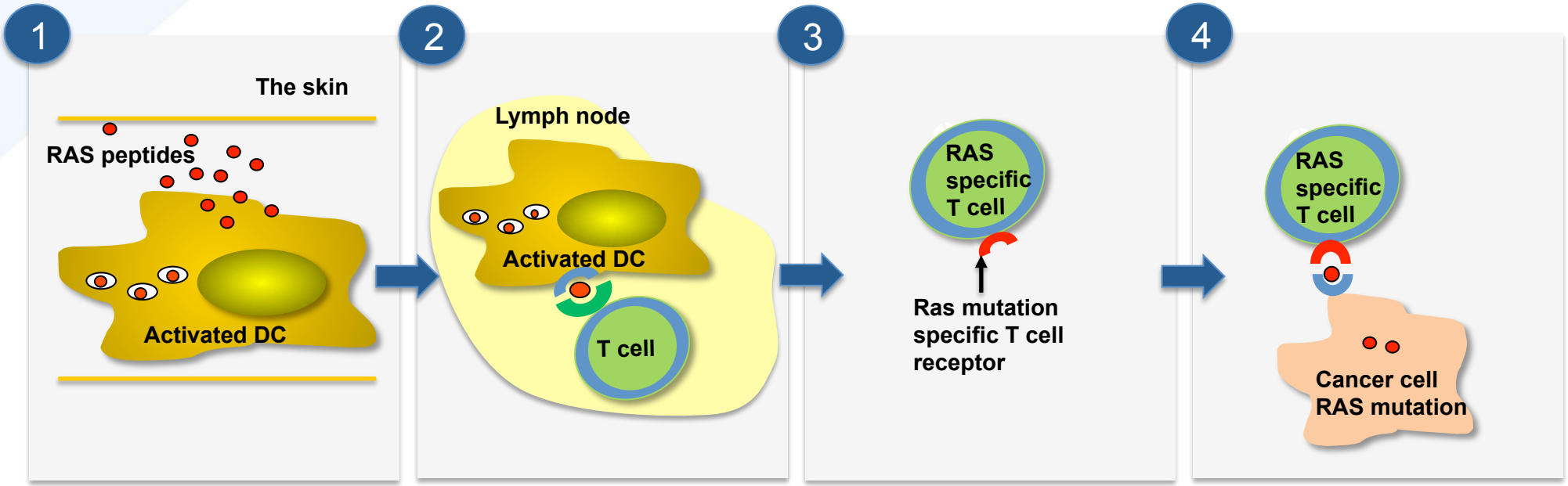
- Commercial kits available for screening of RAS (Qiagen, Roche)
- Currently, screening is routinely done for patients with colorectal cancer
- Example: Patients with colorectal cancer and RAS mutation are taken off current treatment (Erbitux) due to lack of effect

Source: QIAGEN

Targovax´s approach triggers T cells to specifically recognize cells with RAS mutations

How does it work

Mechanism of action



The peptides are injected into the skin together with an adjuvant

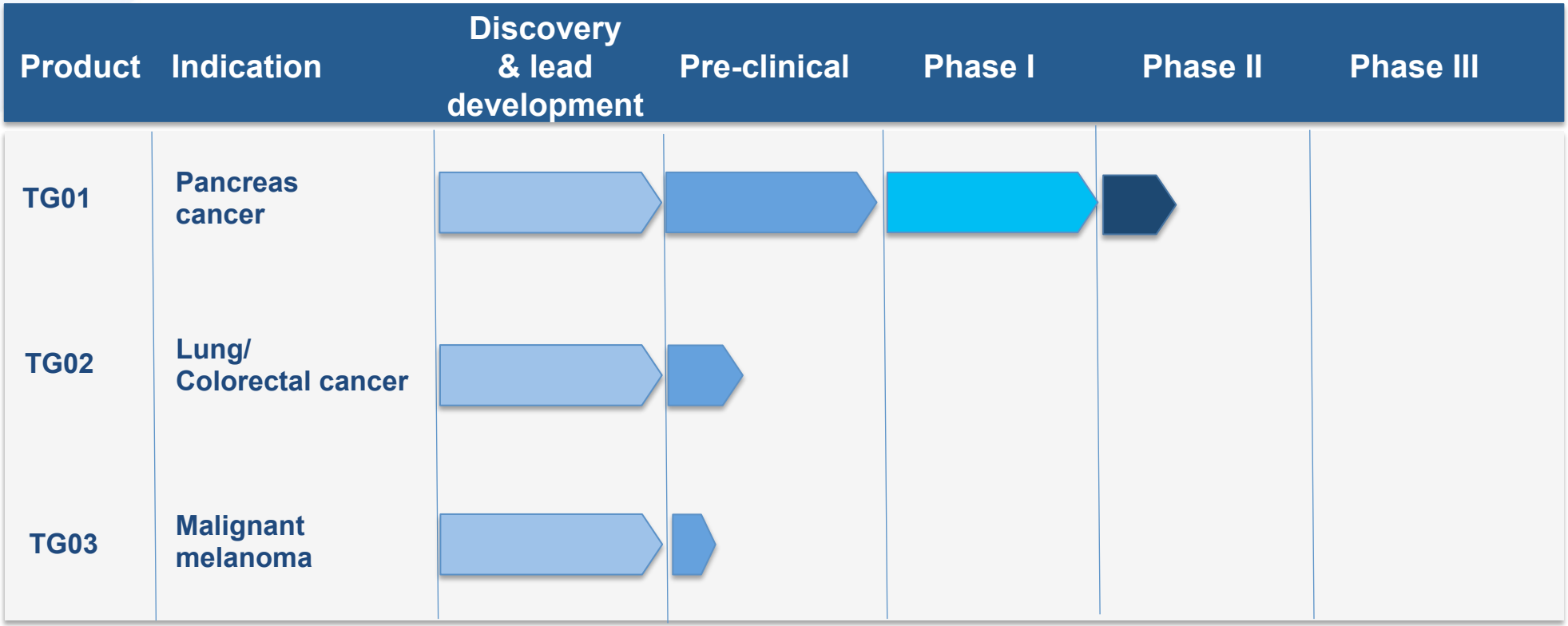
The peptides are taken up by activated dendritic cells that migrate to lymph nodes where they present the peptides to T cells

Subsets of T cells learn to recognize the RAS mutations and enter the circulation of the body

When encountering cancer cells with RAS mutations, the T cells are re-activated to proliferate and trigger immunological killing of the cancer cells

Strong pipeline leveraging on product history and mature lead product TG01

Current pipeline



TG01: Phase I part of phase I/II trial CT TG01-01 in Operated Pancreatic Cancer finished

TG01 is safe and triggers immune responses

- Study objectives
  - Safety of TG01 combined with gemcitabine chemotherapy
  - Immune response to TG01 used in combination with gemcitabine
- Results
  - No serious adverse events related to TG01
    Only one adverse event related to TG01 – skin reactions locally at injection site (expected)
  - All patients (6/6) demonstrated an immune response to TG01 by DTH (skin reaction test)
- Please see appendix for further details on results from the Phase I study

The clinical trial will continue through phase II in order to further investigate immune responses and explore relationships between TG01 immune responses and clinical effects

TG01: Phase II part of phase I/II trial CT TG01-01 ongoing

Investigating clinical effects

- Single arm, open trial (N=18)
- Primary study objectives:
  - Safety of TG01 combined with chemotherapy
  - Immune response to TG01 used in combination with chemotherapy
- Secondary study objectives:
  - Disease Free Survival and Overall Survival (% at 2 years)
- Additional assessments:
  - Immune responses and survival at 12 months, median Disease Free Survival and median Overall Survival
- Internationally renowned clinical centres:
  - The Norwegian Radium Hospital, Christie NHS Foundation Trust in Manchester, UK, Clatterbridge Cancer Centre NHS Foundation Trust in Liverpool, UK

News flow and value inflection points:
Disease free Survival (E2015) and Overall Survival (E2016)

Targovax products covers a substantial market potential

TG01	**PANCREATIC CANCER (90% RAS)** 116 000 patients/year – 16 000-20 000 operable (EU/US)
TG02	**COLORECTAL CANCER (40% RAS)** 1 235 000 patients/year (world wide) **NON SMALL CELL LUNG CANCER (25% RAS)** 1 608 000 patients/year (world wide)
TG03	**MALIGNANT MELANOMA (20% RAS)** >160 000 patients/year (world wide)

New approved cancer medicines have been priced in the range 30 000-60 000 USD

Sources: 2011 – statistics, http://globocan.iarc.fr/Pages/fact_sheets_population.aspx www.cancer.gov

Established, validated technology and lead product protected by Orphan Drug Designation

First product in Phase II

- Lead product TG01 – treatment of operated pancreatic cancer
  - Complete preclinical documentation
  - Phase I completed in combination with chemotherapy – safe and well tolerated by the patients
  - Phase II ongoing
  - Clinical development plan validated by European Medicines Agency
- Immunological Proof of Concept in cancer patients from historical data*
  - RAS mutation specific immune responses (T cells) are induced
  - Immune response indicates a survival benefit for patients
- Production established at phase II level
- Solid foundation for expansion to other indications with products TG02 and TG03

First indication protected by Orphan Drug Status

- TG01 protected by Orphan Drug Designation in US & EU
  - 7 and 10 years market exclusivity for product in indication from date of market approval (see appendix for details)
- Patents covering peptide based immunotherapy targeting RAS:
  - USA: US 5 964 978, (exp. May 2016)
  - Norway: NO309798B1 (exp. Apr 2019)
- Patent application, priority Dec 9th 2013, covering products TG02, TG03 and other products **
- The company works continuously with establishing new patents, patent extensions etc where applicable
- Freedom to operate for TG01, TG02 and TG03 based on analysis from 2014.

* (Gjertsen et al, 2001, Weden et al, 2011)

** The patent application is in early stage of the application process and has not yet been subject to an examination by the EPO. An assessment with respect to patentability will be part of the EPO examination. Patent office: Marks & Clerck, UK

Key financials

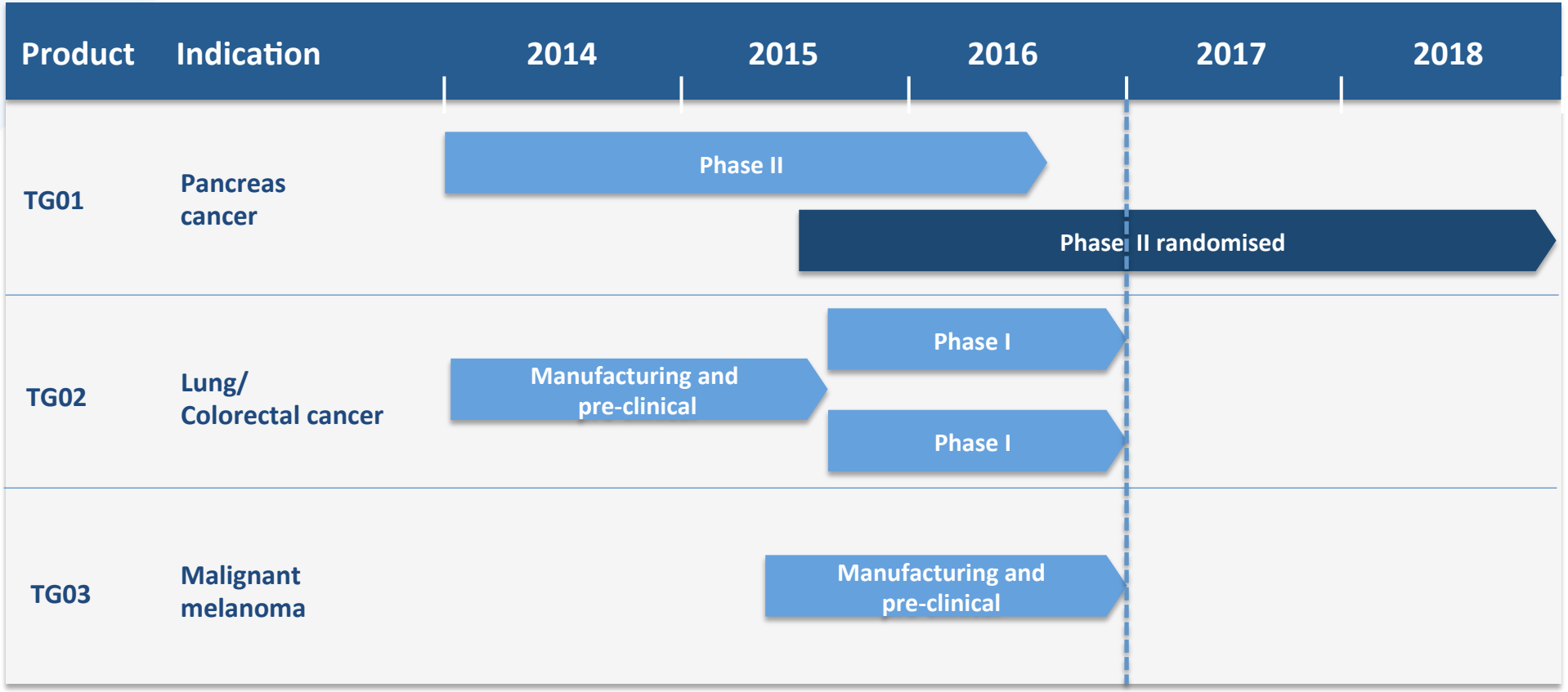
Profit & Loss

	YTD 2014	YTD 2013	2013
OPERATING REVENUES			
Grants	1 095	1 503	4 337
Other revenues	52	33	364
TOTAL OPERATING REVENUES	**1 147**	**1 535**	**4 701**
OPERATING EXPENSES			
Payroll expenses	-2 066	-2 047	-4 721
Other operating expenses	-3 999	-2 640	-7 110
TOTAL OPERATING EXPENSES	**-6 065**	**-4 687**	**-11 831**
OPERATING PROFIT/LOSS	**-4 918**	**-3 152**	**-7 130**
NET FINANCAL ITEMS	**-11**	**-18**	**-15**
PROFIT/LOSS FOR THE PERIOD	**-4 929**	**-3 170**	**-7 144**

Balance sheet

	30 Jun 2014	30 Jun 2013	31 Dec 2013
MNOK			
Receivables	4 519	4 025	5 826
Bank deposits	72 241	2 902	8 370
TOTAL ASSETS	**76 761**	**6 926**	**14 197**
Paid in capital	103 339	20 839	20 839
Retained earnings	-30 170	-16 663	-20 637
TOTAL EQUITY	**73 168**	**4 175**	**201**
Accounts payable	1 711	251	2 657
Public duties payable	291	283	454
Other current liabilities	1 591	2 217	10 884
TOTAL LIABILITIES	**3 592**	**2 751**	**13 996**
TOTAL EQUITY AND LIABILITIES	**76 761**	**6 926**	**14 197**

Product pipeline with clear, planned milestones



Approx. NOK 60 mill.

10 largest shareholdes after capital increase June 2014

	Sum	Share (%)
RADIUMHOSPITALETS FORSKNINGSSTIFTELSE	3 410 589	36,17
DATUM AS	1 162 000	12,32
TIMMUNO AS*	724 650	7,69
PRIETA AS**	720 000	7,64
ALGOT INVEST AS	460 785	4,89
BIRK VENTURE AS	438 657	4,65
TRYGVE SCHIØRBECKS EFTF AS	286 449	3,04
ARCTIC FUNDS PLC BNY MELLON SA/NV	182 000	1,93
PORTIA AS	180 000	1,91
OP-EUROPE EQUITY FUN C/O CITIBANK NA	157 869	1,67
SUM	**7 722 999**	**81,91**

*** Jon Amund Eriksen, ** Gustav Gaudernack**

Strong track record since 2010 and expecting significant value inflection points during 2014-2016

Significant progress made so far

2011
- Orphan Drug status granted in EU and US for TG01
- GMP production established for TG01
- Public grant OFU program MNOK 9

2012
- GMP production for adjuvant GM-CSF from a reliable source
- European Medicines Agency advice supporting the clinical development plan for TG01
- Clinical trial initiated December – Phase I/II in operable pancreatic cancer

2013
- First patient treated
- Public grant BIA MNOK 12.3
- Expanding study to two sites in UK
- Patent application supporting expanded pipeline

2014 (by August)
- Phase I successfully completed with RAS specific immune response in 6/6
- Phase II initiated
- First patient treated UK
- OTC listing
- Start production and pre-clinical development TG02

Anticipated milestones 2014-2016

2014
- Complete enrollment of 18 patients in TG01 phase II trial in operable pancreatic cancer

2015
- Interim analysis disease free survival TG01 phase II (12m)
- Established protocol Phase II randomized TG01
- Complete pre-clinical package TG02
- Initiate TG02 phase I in colorectal and/or lung cancer
- Start production and pre-clinical development TG03

2016
- Complete TG01 phase II operable pancreatic cancer (24m)
- GM-CSF source for Phase III/commercial use concluded
- Complete TG02 phase I in colorectal and/or lung cancer
- Complete pre-clinical package TG03 for phase I
- IPO (1H)

Summary

Progression as planned - Targovax is well positioned with promising clinical data

- Promising pre-clinical and clinical data
- Cancer cell specific immunotherapy
  - Educates the patients' immune system to recognize and kill cancer cells
- No significant side effects
- Induces immune responses which indicates a survival benefit*
- Robust manufacturing of product – low cost of goods
- Stable products with long shelf life and easy logistics
- Product portfolio with a substantial market potential
- Orphan Drug Designation (US/EU) for TG01
- Well positioned for collaborations
- High level of Pharma interest in immunotherapy

* Gjertsen et al, 2001

Supporting materials

THERAPEUTIC CANCER VACCINES

Definitions and glossary of terms

- **Cancer Immunotherapy:** is treatment that uses the body's own immune system to help fight cancer.
- **Peptides** are small proteins. They can be produced chemically in quantities of many kilograms. The RAS peptides are very stable and can be stored for several years.
- **T cells** are the cells of the immune system that defend the host against intracellular changes and infections (virus). By using peptides mimicking special intra cellular changes, like RAS mutations, subset of T cells can be induced that recognize cells with such mutations and that will trigger immunological eradication of these cells**.** T cells also provide immunological memory and are rapidly retriggered upon reappearance of cells with the specific intracellular changes.
- **Dendritic cells (DC)** are specialized immune cells that are present in the skin. When activated DC engulf foreign material and present protein fragments (peptides) to T cells in the lymph nodes where specific T cells are induced.
- **Immune response:** is how the body recognizes and kills virus, bacteria, and substances that appear foreign and harmful.
- **DTH:** a way of measuring an immune response towards a specific antigen. When the immune system has learnt to recognize e.g. the RAS peptides as foreign, the immune response can be measured by using DTH (delayed type hypersensitivity reaction). It is similar to the Pirquet – skin test used to measure effect of tuberculosis vaccination, by applying the foreign material to scratches on the skin and observing the size of swelling / redness.
- **Adjuvant** is used to activate the dendritic cells at the injection site to take up the peptides. Targovax is using GM-CSF (granulocyte macrophage – colony stimulating factor) as adjuvant.
- **GM-CSF** (the adjuvant) is a protein that activates DC by interacting with their GM-CSF receptor. GM-CSF is commercially available (currently for use in phase II).