Unlocking the potential of innovative medicines

PCI Biotech –

DNB Health Care Conference

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Per Walday, CEO

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Highlights

Partnering	▪ Collaborative research with top-10 large pharma initiated ▪ Research work with RXi progressing according to plan
Bile duct study	▪ Patients enrolled into the fourth dose cohort ▪ Potential to progress into Ph II in 1H16
Strategic refocusing	▪ Exploring options for clinical validation of immunotherapy results
Scientific Advisory Committee (SAC)	▪ Network of internationally renowned experts
New location	▪ PCI Biotech moving to Oslo Cancer Cluster Innovation Park

Nucleic acid therapeutics – Research collaborations

Top-10 large pharma company

- Agreement signed in Q3 2015
- Aim is to evaluate synergistic effects between companies' technologies
- One of the global leaders in nucleic acid therapeutics
- Collaborative research funded and initiated
- Data generated in research collaboration to be evaluated- potential for a further partnership

RXi Pharmaceuticals

- RXi Pharmaceuticals (NASDAQ: RXII) discovers and develops innovative therapeutics that address high unmet medical needs- dermatology and ophthalmology
- The collaborative research with RXi progressing according to plan
- Results achieved from this research collaboration to be evaluated- potential for closer collaboration

Clinical study with Amphinex in inoperable bile duct cancer moved into the fourth dose cohort

Why target bile duct cancer?

- **Patient population with high unmet medical need**
- **No approved medical treatments**
- **Easy access with light through routine endoscopic methods**
- **Orphan indication represents a distinct market opportunity**
- **Limited development pipeline**

Attractive due to orphan benefits and absence of satisfying treatments

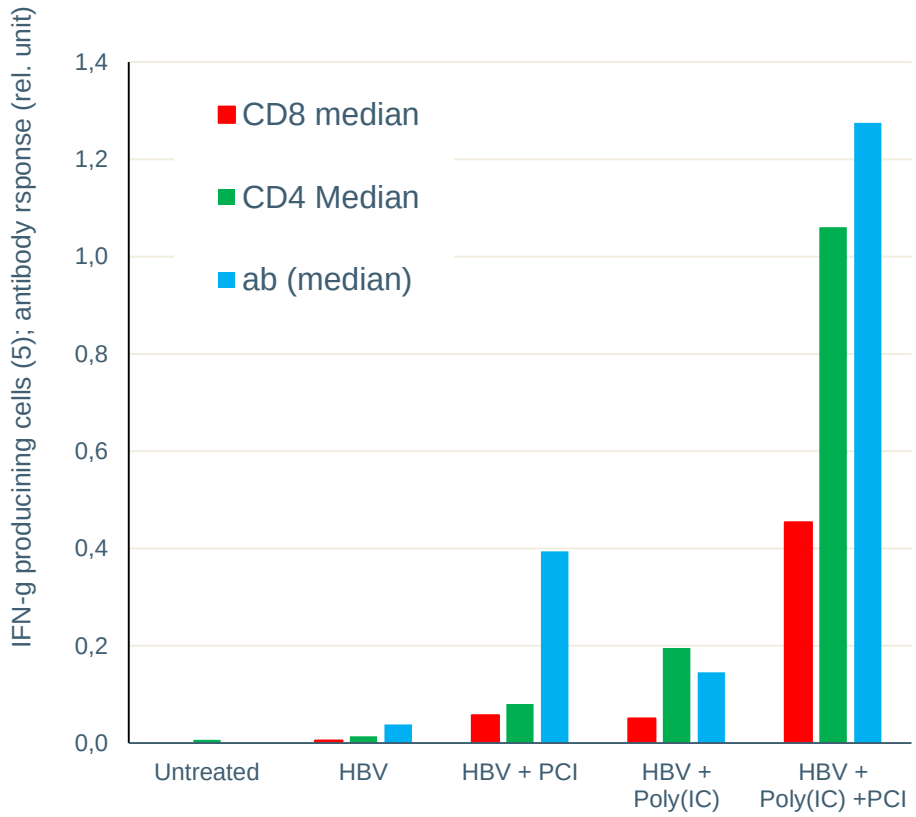
Current status and plans

- Safety driven Phase Ib
- Third dose cohort concluded Aug 2015 – no safety concerns
- Patients enrolled into the fourth dose cohort
- Potential to progress into Phase II in 1H 2016
- Increasing the number of sites in preparation for Phase II
- 5:2 randomisation in Phase II, 35 pts in total

Cohort	Amphinex dose	Light dose
Cohort 1	0.06 mg/kg	15 J/cm
Cohort 2	0.06 mg/kg	30 J/cm
Cohort 3	0.12 mg/kg	30 J/cm
Cohort 4	0.25 mg/kg	30 J/cm

Exploring options for clincial validation of immunotherapy results

PCI can elicit strong responses in all important aspects of immune responses



PCI with HBV protein antigen induces both CD8, CD4 and antibody responses

Scientific Advisory Committee (SAC) – Network of internationally renowned experts

Background / competence / experience	Members	
• Academic luminary and international leading expert in immunology • Broad understanding of vaccines and other immunotherapy modalities		• Prof Christoph Huber (Medical School of the Johannes Gutenberg University in Mainz)
• Oncology opinion leader and expert • Wide international network • Relevant clinical trial experience in collaboration with the pharmaceutical industry		• Prof Jan Vermorken (Antwerp University Hospital)
• Expert and international opinion leader in medical oncology and anti-cancer drug development • Extensive pharmaceutical industry experience		• Prof Andrew Hughes (The Christie, University of Manchester)
• Leading expert in photobiology/photomedicine, cell biology, endocytotic and endo-lysosomal processes, photochemical mechanisms and PDT/PCI		• Prof Kristian Berg (OUS – The Radium Hospital)

Pivotal added value contribution from SAC: Industry experience; Experts within areas that are crucial to PCI development; International opinion leaders with extensive global networks

PCI Biotech

Financial key figures

(In NOK 1,000)	Q3 2015	9M 2015
Other Income	2 415	7 144
Operating costs	11 831	31 234
Operating results	-9 416	-24 090
Financial items	120	546
Comprehensive income	**-9 296**	**-23 544**
Cash & cash equivalents	**53 897**	**53 897**
Total debt	**9 212**	**9 212**
Net cash flow from operations	**-9 538**	**-27 872**

- **Close to MNOK 10 non-dilutive funding for 2015**
- **Financial runway towards end of 2016 at current cost base**

PCI Biotech is well positioned for attractive development opportunities

Main focus going forward:

1. **Progress of the Phase I/II bile duct cancer study**
2. **Clinical validation of immunotherapy results**
3. **Partnering agreements and alliance progress**

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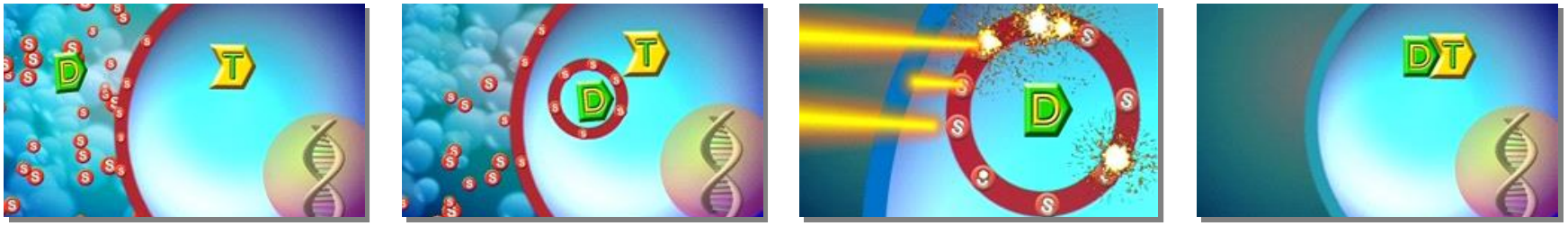
Supporting slides

PCI Biotech at a glance

Unlocking the potential of innovative medicines

- A listed (PCIB:NO) cancer-focused biotech company
- Photochemical internalisation (“PCI”) technology, originating from the Norwegian Radium Hospital
- Clinical Program
  - Phase I/II with the photosensitiser Amphinex® for the orphan indication inoperable bile duct cancer
- Pre-clinical programs
  - Vaccine delivery technology that provides strongly enhanced T-cell responses
  - Efficient delivery of macromolecules, such as nucleic acid therapeutics

PCI deliver drugs into cells through illumination



D The active molecule

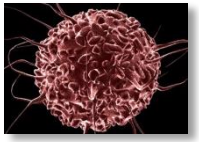
S The PCI component

T The target

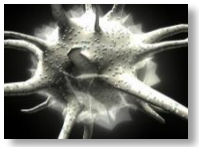
PCI technology – enabling drugs to cover additional areas of unmet medical need

Existing & innovative treatments

Cells



Cancerous cell



Dendritic cell

Active ingredient
(trapped in endosome)

- Small molecules
- siRNA/mRNA
- Antibody targeted drugs
- Peptides
- Antigens

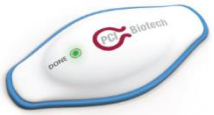
PCI enhancement technology

Photosensitizer
(Amphinex™)

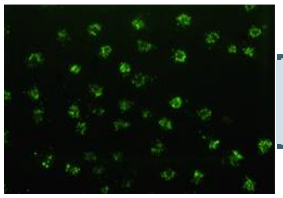
TPCS2a

Light source

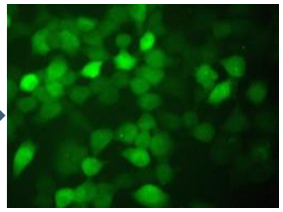
Red light



Blue light



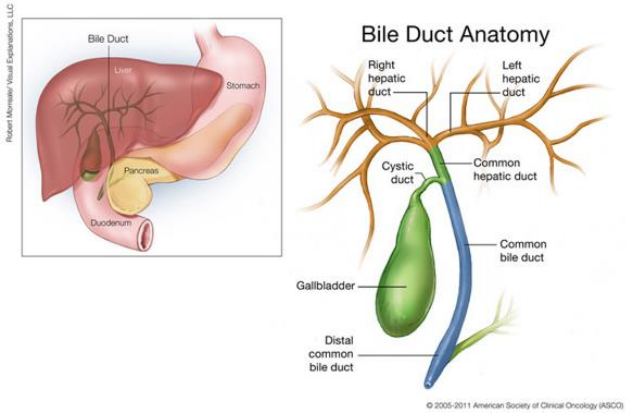
Endosomal escape
Release of drug in cells



PCI – a versatile technology with a pipeline of partnering opportunities

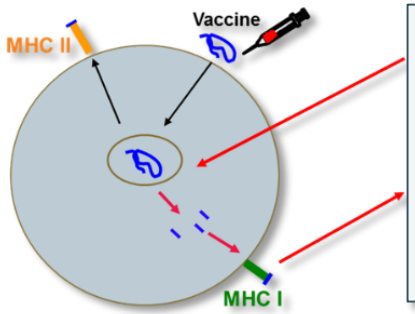
1 Local cancer treatment

- Bile duct cancer



2 PCI vaccination technology

- Focus on therapeutic vaccination

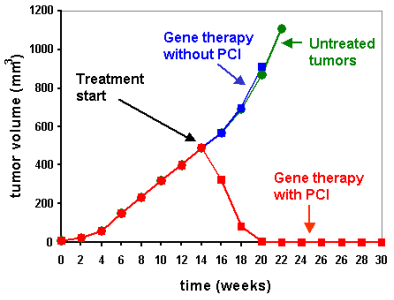


PCI – induce presentation on MHC class I

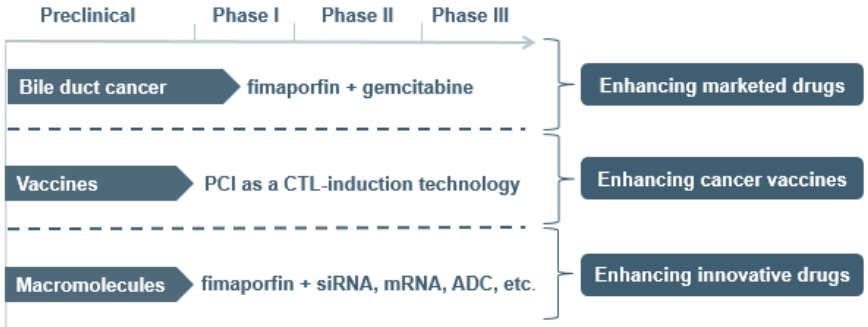
- Make it possible to achieve cytotoxic T-cell response with protein/ peptide vaccines
- Can solve a key challenge for many vaccine approaches

3 PCI macromolecule delivery

- siRNA & other oligos
- Gene therapy
- Immunotoxins



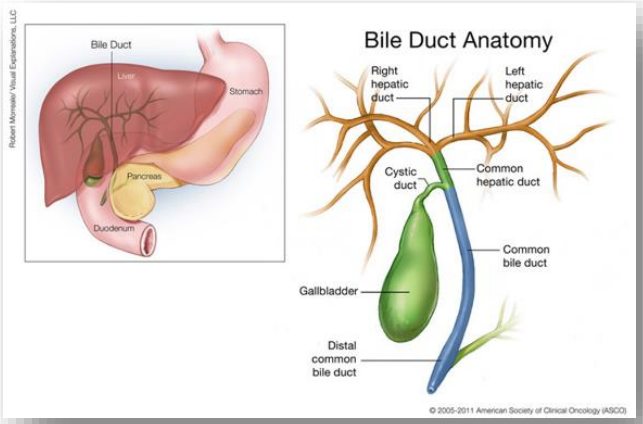
PCI development pipeline



Bile duct cancer – introduction and clinical study design

Introduction to bile duct cancer

- Cancer affecting the cell lining of the bile duct (Medical term: Cholangiocarcinoma)
- Orphan disease – incidence rate of 1-2 per 100,000 in the western world
- Five-year survival rate of less than 5%, and 0% when inoperable
- Incidence and mortality rates are increasing worldwide



Summary of Study Design	
Cancer type	Bile duct (Cholangiocarcinoma)
Phase	Ib/II
Photosensitizer	Amphinex® (PCIB)
Drug	Gemcitabine (Cisplatin)
Light source	Red laser 652 nm (PCIB)
Fixed variables	Gemcitabine and Cisplatin
Variables	Amphinex® and/or light dose
Purpose of study	Open-label, multi-centre study to assess the safety and efficacy of a single treatment of Amphinex® induced PCI of gemcitabine, followed by systemic cisplatin/ gemcitabine. All patients are stented. Phase I to find light and Amphinex® dose. Phase II randomized to compare PCI vs. stenting alone
Patient description	Locally advanced inoperable bile duct cancer
Treatment modality	Intraluminal illumination
Patient sample size	Up to 45 patients
Primary endpoint:	Progression free survival

Bile duct cancer – an orphan indication with a sizeable market potential

Immediate target market is as first line treatment

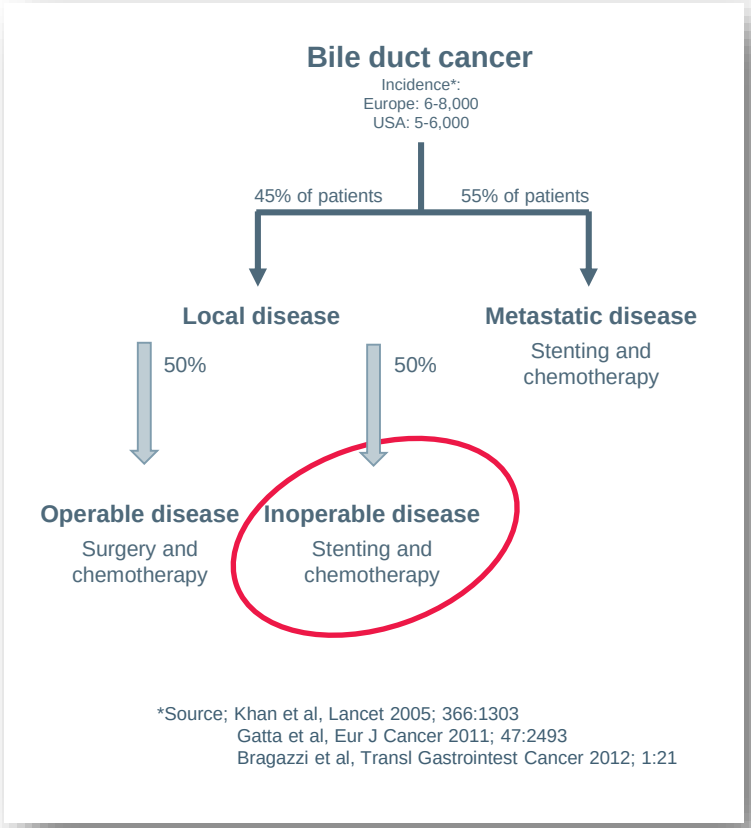
- Immediate target is inoperable patients with local disease
- Approximately 3,000 assumed to be eligible for PCI treatment
- Possible upside potential in metastatic disease

High price potential

- Lack of approved medicinal treatment options
- Orphan indication implies a high price

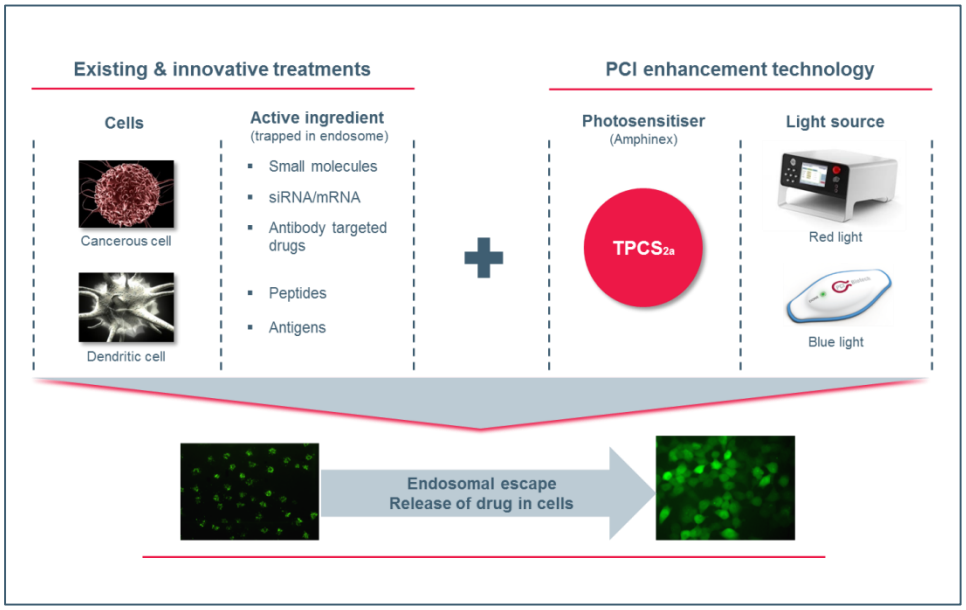
Potential significant majority share of the market

- Anticipated benefits
  - ➢ No competing marketable treatment alternatives
  - ➢ Greater efficacy due to local chemotherapy boost
  - ➢ Easy light access through established standard procedures



Unlocking the true potential of new treatment paradigms

Enhancement of therapeutic vaccination and delivery of macromolecules



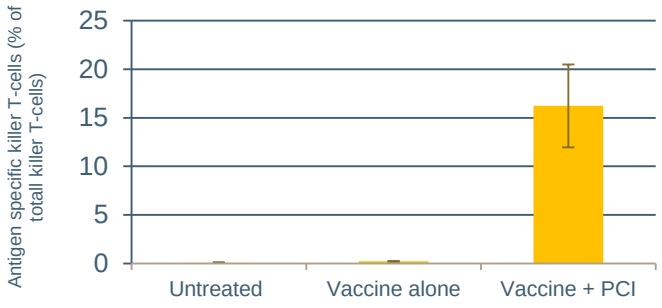
- PCI is a clinically proven endosomal escape technology that may realise the true therapeutic benefit of innovative medicines
- Strong preclinical efficacy evidence
  - ➢ Potentiation of responses considered key for effective **therapeutic vaccination**
  - ➢ Effective localised delivery of a range of **macromolecules**
- Value will be captured through licensing deals and strategic R&D alliances – currently in discussion with potential partners

PCI may realise additional therapeutic potential of innovative medicines and increase their coverage of unmet need in certain disease areas

Therapeutic cancer vaccines – PCI as a powerful vaccine delivery technology

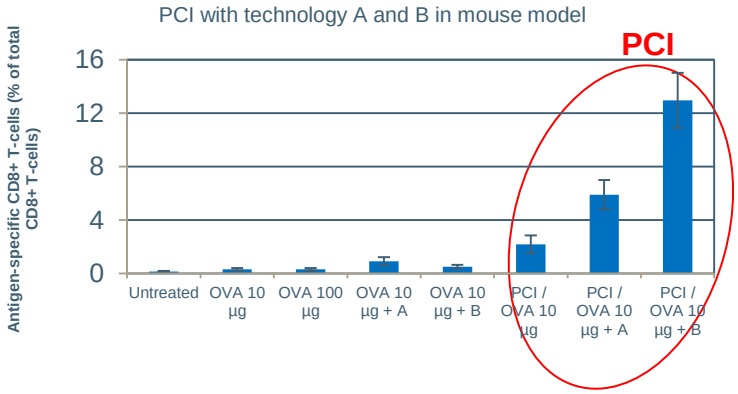
1 **An effective immune-potentiator,**

PCI *in vivo* vaccination in mouse model

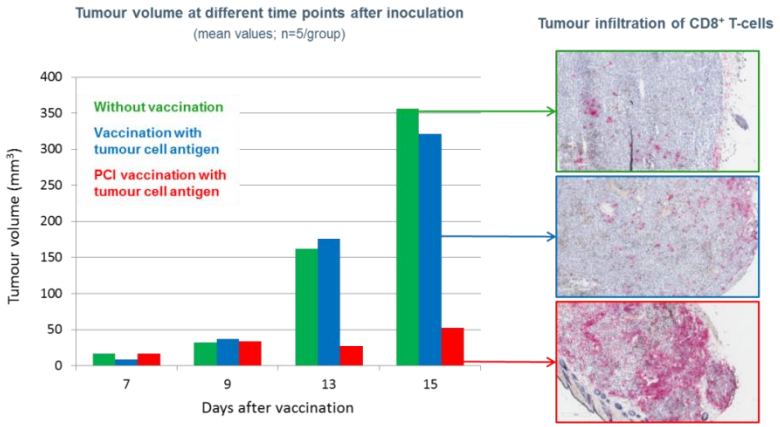


2 **that works in synergy with state-of-the-art vaccine technologies**

PCI with technology A and B in mouse model



3 **and translates into therapeutic effect in disease models.**



PCI vaccination technology – competitive advantages and user-friendly solutions

Clinical safety and preclinical efficacy, combined with a comprehensive patent estate on PCI-mediated CTL-induction (products, uses and devices)

User-friendliness – fully integrated treatment without waiting time: fimaporfin one component of vaccine and patent on integrated disposable band aid-like device that ensure automatic optimal light activation

Safety – fimaporfin well tolerated in Phase I at high systemic (i.v.) doses

Stability – fimaporfin can be autoclaved and is stable at room temperature, also in solution

Innovation – Unique mode of action; fimaporfin provides CTL-induction by MHC class I antigen presentation in dendritic cells and macrophages

Cost effectiveness – Simple and cost effective synthesis

Broad applicability – Peptide/protein antigens and particulate antigen formulations; therapeutic & prophylactic vaccination; *in vivo & ex vivo*