



# PCI BIOTECH

Unlocking the potential of innovative medicines

Q4 2015 PRESENTATION

February 9, 2016

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# PCI BIOTECH

## ▶ Important notice and disclaimer

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# HIGHLIGHTS

▶ Q4 2015

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## Bile duct study

- Completed dose escalation in the bile duct cancer study, with promising early signs of efficacy
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## Strategic refocusing

- Progressing the vaccination technology towards clinical validation
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## Partnering

- Research agreement signed with Ultimovacs – currently three active research collaborations with commercial entities
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## Moved to OCCI

- Enables us to further develop and capitalise on the close cooperation with the Radium Hospital where the PCI technology originated
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# PCI BIOTECH AT A GLANCE

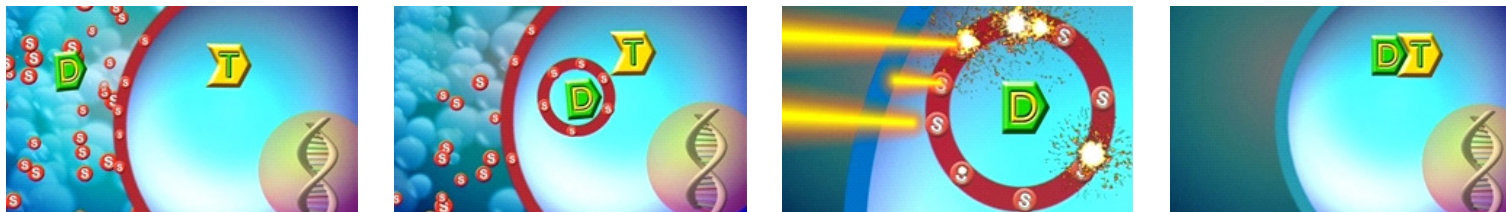
## ▶ Unlocking the potential of innovative medicines

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

### PCI deliver drugs into cells through illumination

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The active molecule

The PCI component

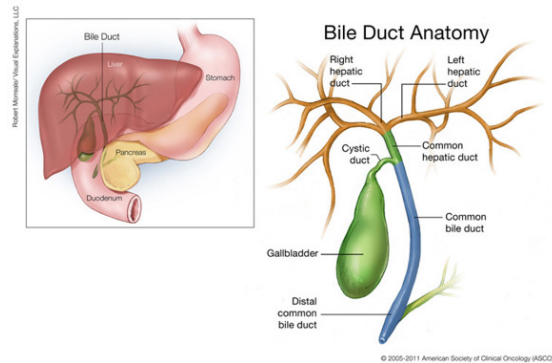
The target

# PCI BIOTECH

▶ Three promising development areas

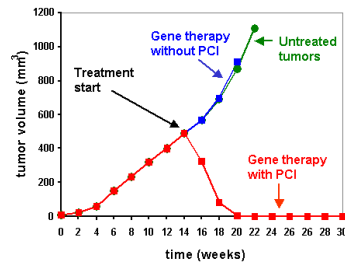
## 1 Local cancer treatment

- Bile duct cancer



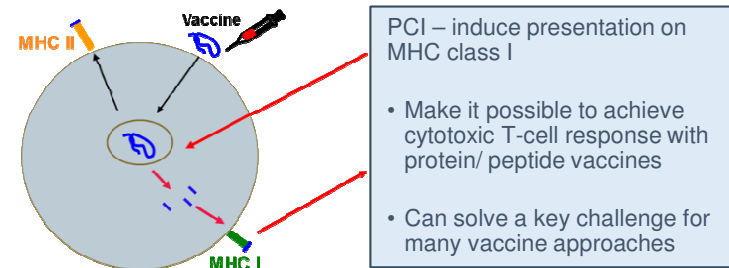
## 3 PCI macromolecule delivery

- siRNA & other oligos
- Gene therapy
- Immunotoxins

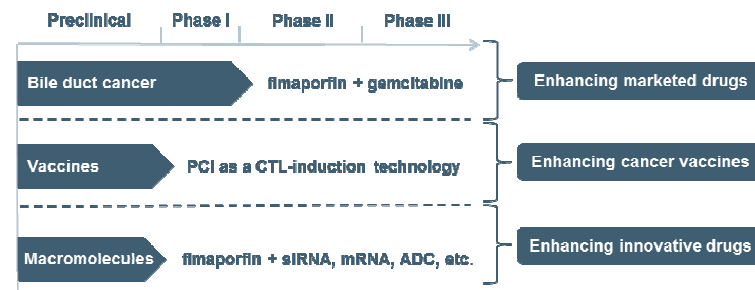


## 2 PCI vaccination technology

- Focus on therapeutic vaccination

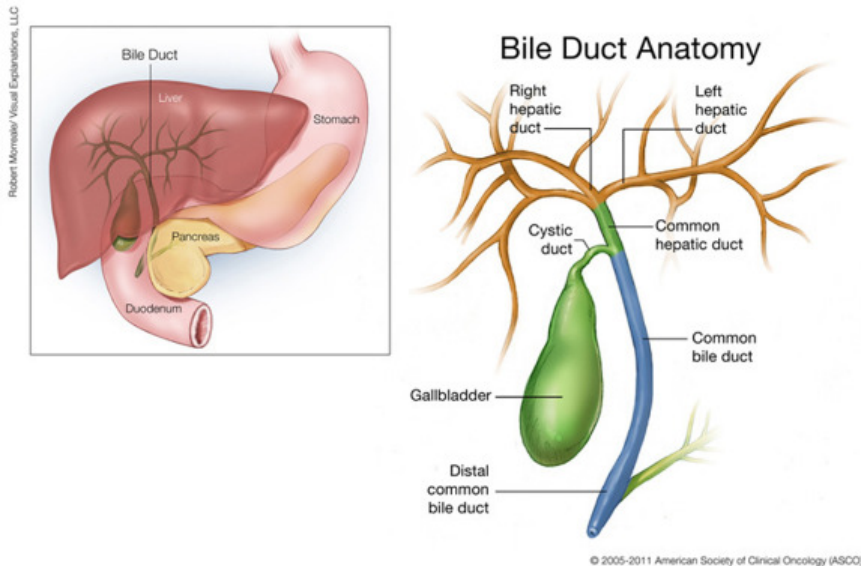


## PCI development pipeline



# BILE DUCT CANCER

- ▶ A rare but fatal disease



- ▶ **Five year survival less than 5%**
- ▶ **Remarkable resistance to chemotherapy**
- ▶ **Estimated market potential of up to USD 500m for efficacious treatment**
- ▶ **Phase I/II trial ongoing with fimaprofin**
  - combination with gemcitabine
  - open-label, multi-center trial in up to 45 patients
  - activation of fimaprofin by intraluminal illumination

# BILE DUCT CANCER

- ▶ Progressing into Phase II with promising early signs of efficacy

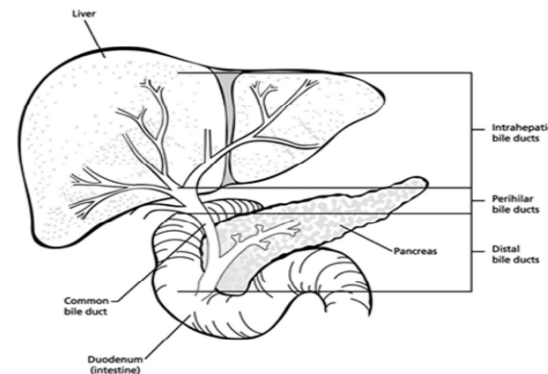
## Why target bile duct cancer?

- ▶ Significant inoperable patient population with high unmet local treatment need
- ▶ No approved medical treatments
- ▶ Limited development pipeline
- ▶ Active chemotherapy enhanced by PCI
- ▶ Easy access with light through routine endoscopic methods

*Attractive due to orphan benefits and absence of satisfying treatments*

## Current status and plans

- ▶ Safety driven Phase Ib completed
- ▶ Fourth dose cohort concluded Jan 2016 – no safety concerns
- ▶ Promising early signs of efficacy in previous dose cohort
- ▶ Progressing into Phase II
- ▶ Increasing the number of sites for Phase II



# Clinical phase I/II study in bile duct cancer

▶ Phase I – Preliminary response data

6 months radiology (CT) data from 3 dose cohorts:

Cohort	Amphinex dose	Light dose	PD	SD	PR	CR	NA*	
Cohort 1	0.06 mg/kg	15 J/cm	1	1			1	
Cohort 2	0.06 mg/kg	30 J/cm		3				
Cohort 3	0.12 mg/kg	30 J/cm		1	1	1		
Cohort 4	0.25 mg/kg	30 J/cm	Not yet available – subjects on-going					

**PD: Progressive disease**  
(>25% growth)

**SD: Stable Disease**

**PR: Partial Response**  
(>30% shrinkage)

**CR: Complete Response**  
(no visible tumour)

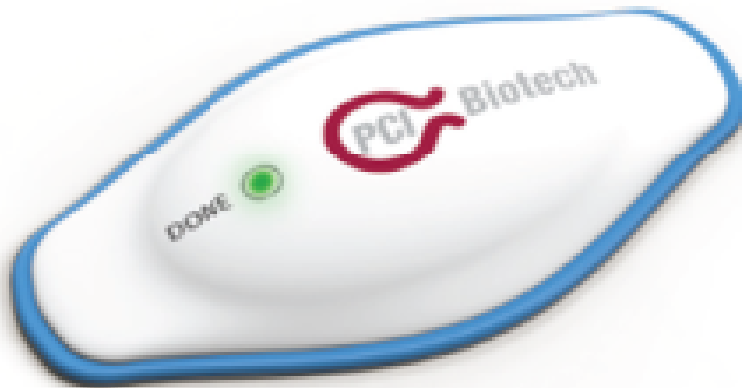
\* Not measurable / Not evaluable



# PCI VACCINATION

▶ Opportunity to play a key role in second generation immunotherapy

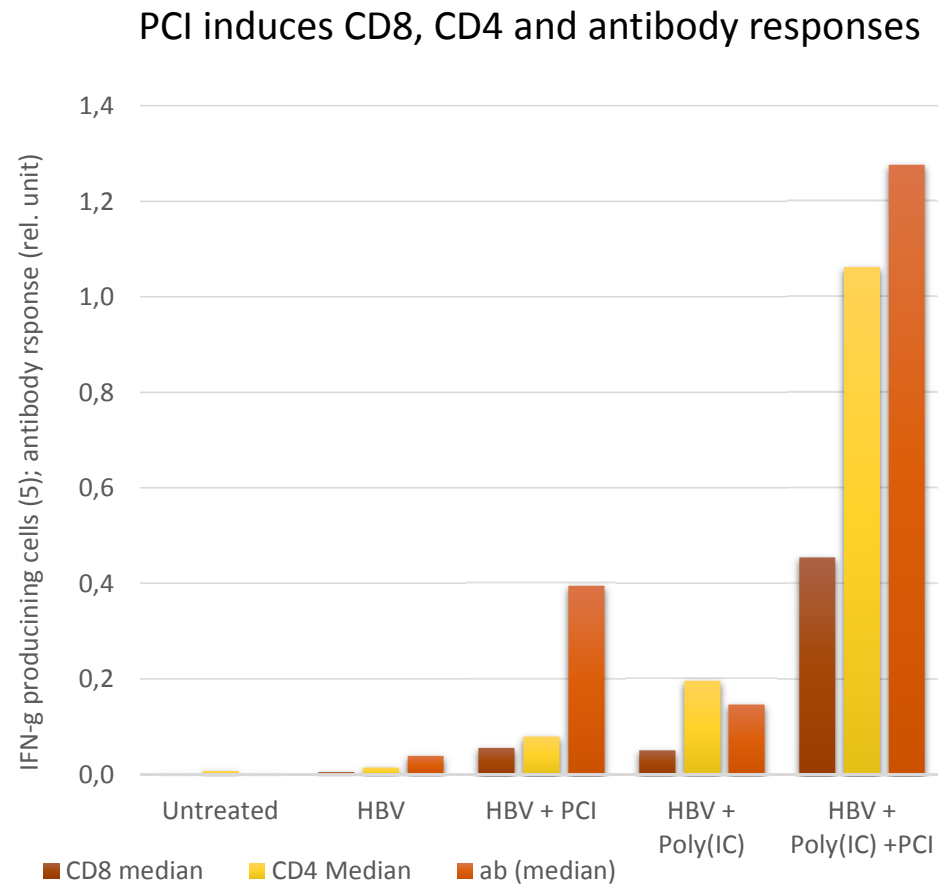
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- ▶ Unique mode of action
  - indication of CTL-induction by MHC class I antigen presentation in dendritic cells and macrophages
- ▶ Broad applicability
  - peptide and protein antigens
  - particulate antigen formulations
  - prophylactic & therapeutic vaccination
- ▶ Safety confirmed in Phase I studies
- ▶ Excellent stability
  - stable at room temperature
  - stable in solution
  - can be autoclaved
- ▶ Cost effective synthesis

# PCI VACCINATION

▶ Improving the efficacy of therapeutic cancer vaccines



Promising data from preclinical testing:

- ▶ Elicit strong responses in all important aspects of immune responses
- ▶ Induce antigen-specific killer T-cells
- ▶ Works in synergy with other state-of-the-art vaccine enhancement technologies
- ▶ Opportunity for clinical validation

# RESEARCH COLLABORATIONS

- ▶ Three active collaborations within nucleic acid therapeutics and vaccination

## Top-10 large pharma company

- Agreement signed in 3Q 2015
- 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



- Agreement signed 2Q 2015
- Rxi Pharmaceuticals listed on Nasdaq (NASDAQ: RXII)
- Discovers and develops innovative therapeutics within dermatology and ophthalmology
- Results achieved from this research collaboration to be evaluated-potential for closer collaboration

## Ultimovacs



- Agreement signed 1Q 2016
- Ultimovacs AS, Norwegian immunotherapy company
- Developing UV1, a therapeutic cancer vaccine directed against human telomerase
- Results from this research collaboration to be evaluated-potential for closer collaboration

# FINANCE

## ▶ Key financial figures

<i>(In NOK 1,000)</i>	<b>Q4 2015</b>	<b>Q4 2014</b>	<b>FY 2015</b>	<b>FY 2014</b>
Other Income	3 323	1 542	10 467	7 297
Operating costs	11 862	11 807	43 096	43 769
Operating results	-8 539	-10 265	-32 629	-36 472
Financial items	160	59	707	632
<b>Comprehensive income</b>	<b>-8 379</b>	<b>-10 206</b>	<b>-31 922</b>	<b>-35 840</b>
<b>Cash &amp; cash equivalents</b>	<b>49 249</b>	<b>15 754</b>	<b>49 249</b>	<b>15 754</b>
<b>Total debt</b>	<b>12 114</b>	<b>11 269</b>	<b>12 114</b>	<b>11 269</b>
<b>Net cash flow from operations</b>	<b>-4 648</b>	<b>-3 891</b>	<b>-31 974</b>	<b>-30 862</b>

- ▶ More than mNOK 10 in non-dilutive funding for FY 2015
  - appr. 1/4 of burn rate covered through public grants
- ▶ Positive impact on net cash flow in Q4 through SkatteFUNN receivables
- ▶ Financed through 2016, at current cost base

# PCI BIOTECH

- ▶ Well positioned for attractive development opportunities
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## **Main focus going forward:**

- ▶ Progress of the Phase I/II bile duct cancer study
  - Entering Phase II
- ▶ Clinical validation of the immunotherapy results
  - Progress towards first in man study
- ▶ Partnering and alliance progress
  - Further collaborations and agreements

# POTENTIAL VALUE INFLECTION POINTS

- ▶ Enhancing the value of an investment in PCI
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## 1 Local cancer treatment

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- ▶ Data from bile duct cancer trial
- ▶ Initiation of phase II trial
- ▶ Data from phase II trial
- ▶ Partnering deal

## 2 PCI vaccination

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- ▶ Initiation of PoC trial in humans
- ▶ Data from PoC trial
- ▶ R&D collaborations
- ▶ Partnering deals

## 3 Nucleic acid therapeutics

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- ▶ Progress of ongoing research collaborations
- ▶ New research collaborations
- ▶ Partnering deals

# PCI BIOTECH HOLDING ASA

## ▶ Enquiries

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