



# PCI BIOTECH

Unlocking the potential of innovative medicines

Q1 2018 PRESENTATION

May 8, 2018

Per Walday, CEO

Ronny Skuggedal, CFO



# PCI BIOTECH

## ► Important notice and disclaimer

---

This presentation may contain certain forward-looking statements and forecasts based on uncertainty, since they relate to events and depend on circumstances that will occur in the future and which, by their nature, will have an impact on PCI Biotech's business, financial condition and results of operations. The terms "anticipates", "assumes", "believes", "can", "could", "estimates", "expects", "forecasts", "intends", "may", "might", "plans", "should", "projects", "programmes", "will", "would" or, in each case, their negative, or other variations or comparable terminology are used to identify forward-looking statement. There are a number of factors that could cause actual results and developments to differ materially from those expressed or implied in a forward-looking statement or affect the extent to which a particular projection is realised. Factors that could cause these differences include, but are not limited to, implementation of PCI Biotech's strategy and its ability to further grow, risks associated with the development and/or approval of PCI Biotech's products candidates, ongoing clinical trials and expected trial results, the ability to commercialise fimaporfin (Amphinex<sup>®</sup>), technology changes and new products in PCI Biotech's potential market and industry, the ability to develop new products and enhance existing products, the impact of competition, changes in general economy and industry conditions and legislative, regulatory and political factors. No assurance can be given that such expectations will prove to have been correct. PCI Biotech disclaims any obligation to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise.

The reservation is also made that inaccuracies or mistakes may occur in this information given about current status of the Company or its business. Any reliance on the information is at the risk of the reader, and PCI Biotech disclaims any and all liability in this respect.

# HIGHLIGHTS

► Q1 2018

---

**fima** *CHEM*

- Encouraging interim overall survival data from Phase I in bile duct cancer
  - Preparations for pivotal phase progressing towards initiation 2H'18
- 

**fima** *VACC*

- Promising interim clinical results from Phase I suggesting enhancement of several parameters of importance for vaccination
  - Completed dose-finding part of the Phase I study
- 

**fima** *NAC*

- Extension of the top-10 pharma collaboration
- 










**Corporate**

- Oslo Børs listing, as a transfer from Oslo Axess (subsequent event)

# PCI BIOTECH AT A GLANCE

## ▶ Unlocking the potential of innovative medicines

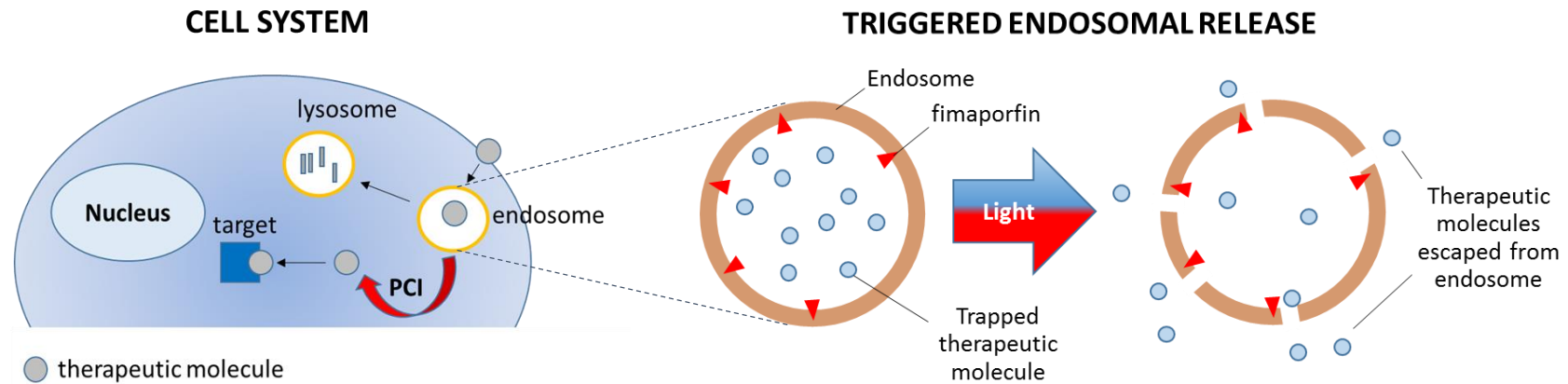
- ▶ A listed (PCIB:NO) cancer-focused biotech company
- ▶ Photochemical internalisation (“PCI”) technology, originating from the Oslo University Hospital – the Radium Hospital

| Programme   | Indications / Therapeutics  | Preclinical  | Phase I | Phase II | Pivotal | Status  |
|---|---|--|---------|----------|---------|---|
|  <b>fimaCHEM</b> |  <i>Bile duct cancer / gemcitabine</i> |   |         |          |         | <ul style="list-style-type: none"> <li>- Promising Phase I results for treatment in the orphan indication bile duct cancer</li> <li>- Plan to initiate pivotal study 2H 2018</li> </ul> |
|  <b>fimaVACC</b> |  <i>Therapeutic cancer vaccines</i>    |   |         |          |         | <ul style="list-style-type: none"> <li>- Ongoing Phase I study in healthy volunteers</li> <li>- Promising initial immune results</li> <li>- One active R&amp;D collaboration</li> </ul> |
|  <b>fimaNAC</b> |  <i>Nucleic acid therapeutics</i>     |  |         |          |         | <ul style="list-style-type: none"> <li>- Four active R&amp;D collaborations</li> </ul>  |

*An oncology focused company with three well differentiated assets*

# PCI TECHNOLOGY

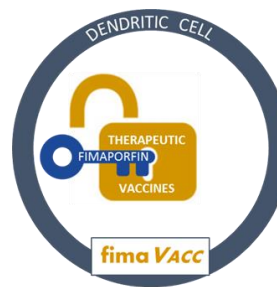
► Enabling drugs to reach intracellular therapeutic targets



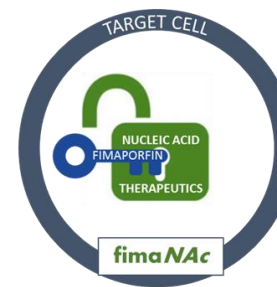
**PCI – the solution to a key challenge for several modalities**



Enabling approved drugs to fulfil unmet local treatment need



Enhancing cellular immune responses important for therapeutic effect

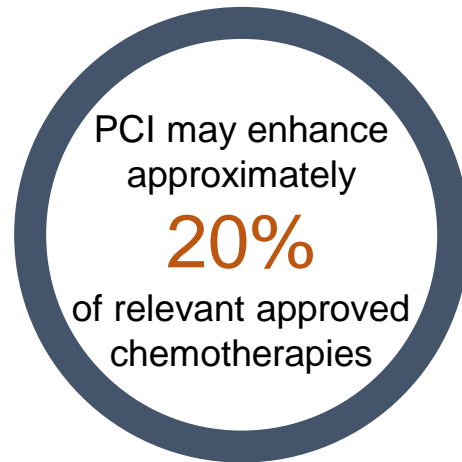


Providing a delivery solution for nucleic acid therapeutics

# THE SOLUTION TO A KEY CHALLENGE

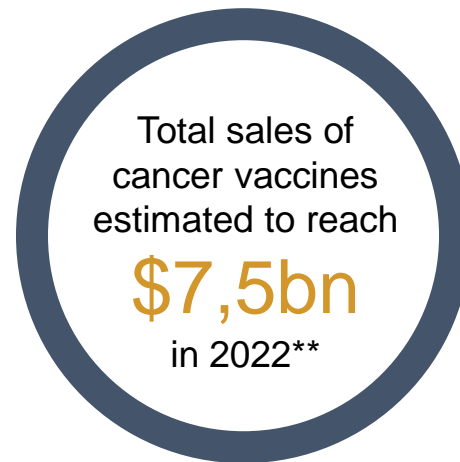
## ▶ Three well-defined development programmes

### fimaCHEM



- ▶ First-in-man study published in Lancet Oncology\*
- ▶ Promising tumour responses in Phase I in inoperable extrahepatic bile duct cancer
- ▶ Incidence close to 15,000 (Eur.+US), with ≈3,000 assumed eligible for **fimaCHEM**
- ▶ Possible upside in distal and metastatic disease, and in Asia
- ▶ Orphan indication with high price potential

### fimaVACC



- ▶ Expected market growth largely driven by therapeutic vaccine combinations with checkpoint inhibitors
- ▶ Aim is to out-license the technology on non-/semi-exclusive basis
- ▶ Opportunity to develop own therapeutic vaccination products

### fimaNAC



- ▶ Estimated sales of \$18bn in 2030\*\*\* (RNAi alone)
- ▶ Opportunistic collaborative approach
- ▶ Aim is to out-license the technology on non-/semi-exclusive basis

\* Lancet Oncology (2016) **17**(9): p1217–1229

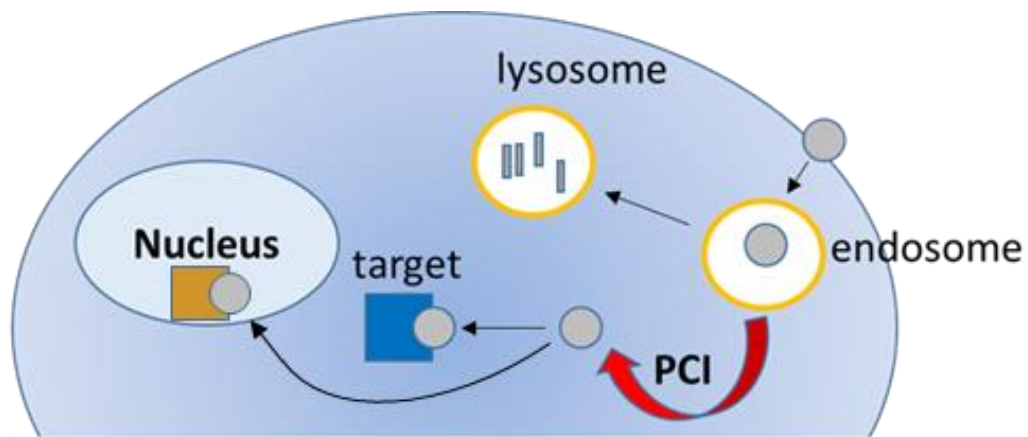
\*\* GBI Research (2016) Global Cancer Vaccines Market to 2022

\*\*\* Research and Markets (2015) RNAi therapeutics market

# PCI TECHNOLOGY

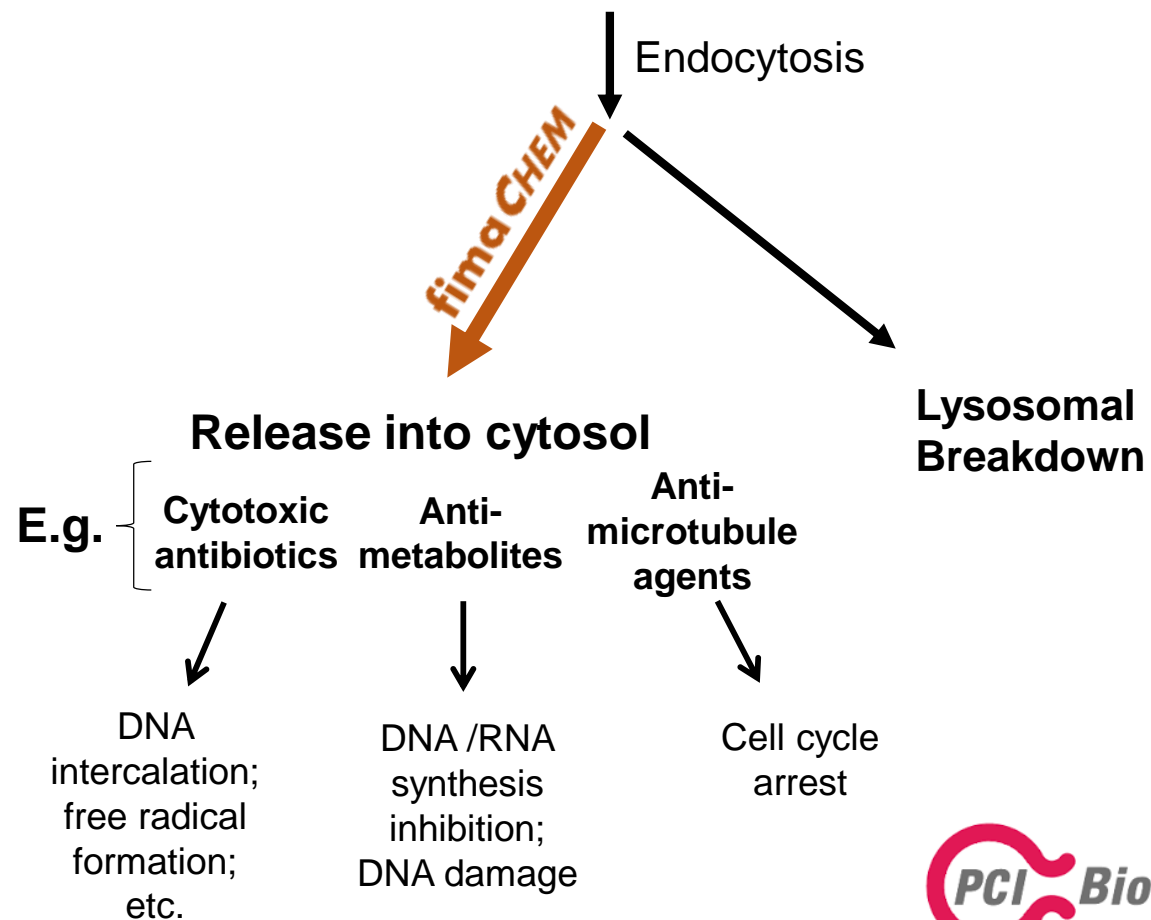
► fimaCHEM – mode of action

## Cancer cell



● chemotherapy

## Chemotherapeutics



# BILE DUCT CANCER

## ▶ Excellent fit between medical need and **fimaCHEM**

- ▶ Orphan indication, yearly incidence rate of 1-2 per 100,000 in the western world – higher in Asia
- ▶ Five-year survival rate of less than 5% and almost 0% when inoperable
- ▶ Average survival inoperable: ≈12 months
- ▶ Current management
  - Surgery
    - Only potentially curative treatment
    - Less than 1/3 are resectable at presentation
  - Stenting
    - **Endoscopic** stenting for palliative biliary drainage
  - Chemotherapy
    - No approved chemotherapy
    - Recommended: **gemcitabine** and cisplatin

### Enhancing the active and recommended chemotherapy

- Combination therapy with gemcitabine and cisplatin is recommended
- Gemcitabine is significantly enhanced by **fimaCHEM**
- Enhancing systemic therapy locally

### Easy illumination through standard endoscopic methods

- Patients are treated with endoscopic methods (ERCP) for diagnosis and stenting
- Optic fibre and illumination easily included in the ERCP procedure

### Boosting chemotherapy effect where it is most needed

- Tumours tend to block the bile duct
- Liver function is often affected
- Biliary drainage is key for patient treatment and survival

### Inducing immunogenic tumour cell death

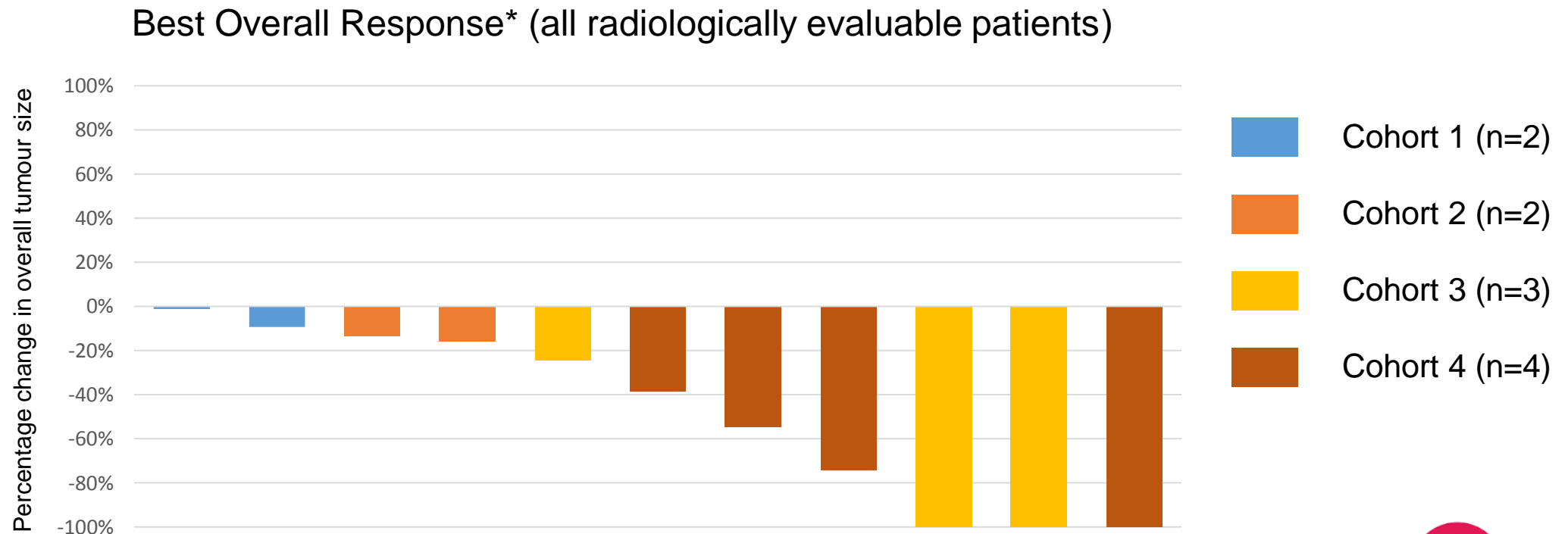
- Preclinical and clinical data supports the notion of potential abscopal effects with **fimaCHEM**
- May be ideal for combination with checkpoint inhibitors



# BILE DUCT CANCER – CLINICAL PHASE I/II STUDY

## ► Encouraging early signs of efficacy in Phase I

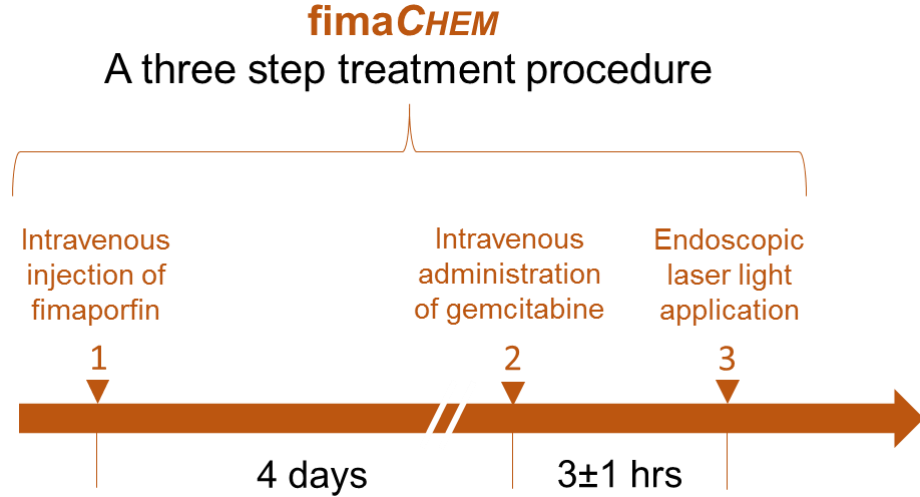
- Interim average overall survival (OS) of all 16 patients in Phase I was 17.4 months per March 2018, with 25% of the patients still being alive. Median OS ended at 14.4 months.



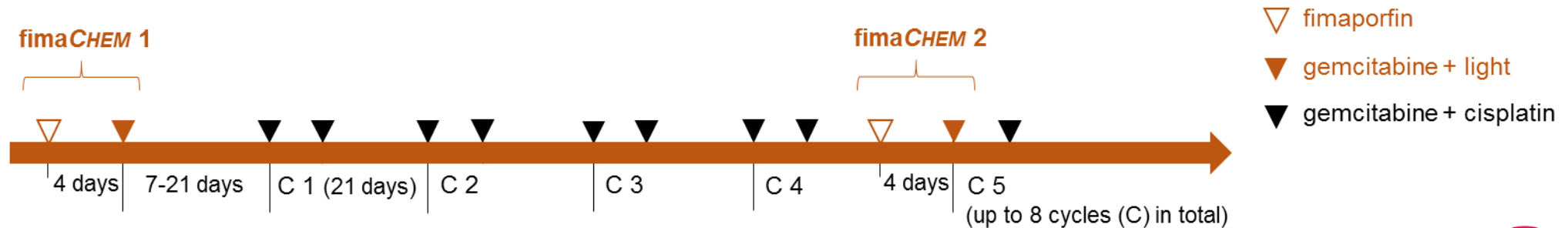
\*Cohort 1 & 2: local read; Cohort 3 & 4: central read

# BILE DUCT CANCER – PHASE I EXTENSION STUDY

▶ Repeating the **fimaCHEM** treatment with the aim to further enhance efficacy



- ▶ Exploring safety of repeating the **fimaCHEM** treatment in an extension to Phase I, which may allow for repeated treatment in a potential pivotal Phase II study
- ▶ The study is progressing according to plan and done in parallel with other preparations for the next phase



# INOPERABLE EXTRAHEPATIC BILE DUCT CANCER

## ▶ Status and strategy going forward

---

### ▶ Orphan designation

- Granted in both the US and EU, recognising the medical need and potential therapeutic benefits

### ▶ Phase I dose-escalation completed with good tolerability and promising early signs of efficacy

- Tumour shrinkage in almost all radiologically evaluable patients
- Encouraging interim overall survival data, with 25% of patients still alive

### ▶ Fastest way to market determined through regulatory interactions with authorities

- Single randomised pivotal study with potential for accelerated / conditional approval based on interim analysis

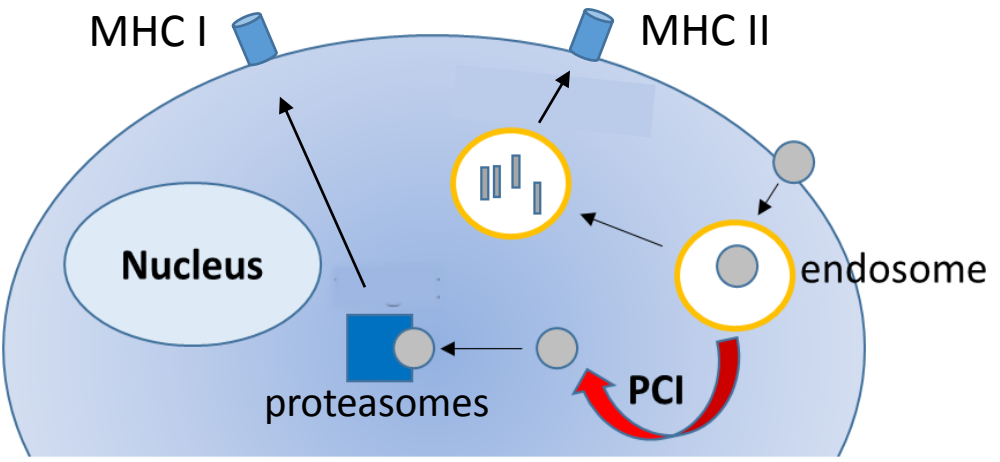
### ▶ Preparations for pivotal phase progressing towards initiation 2H 2018

- Full study design to be announced upon completion of clinical advisory interactions

# PCI TECHNOLOGY

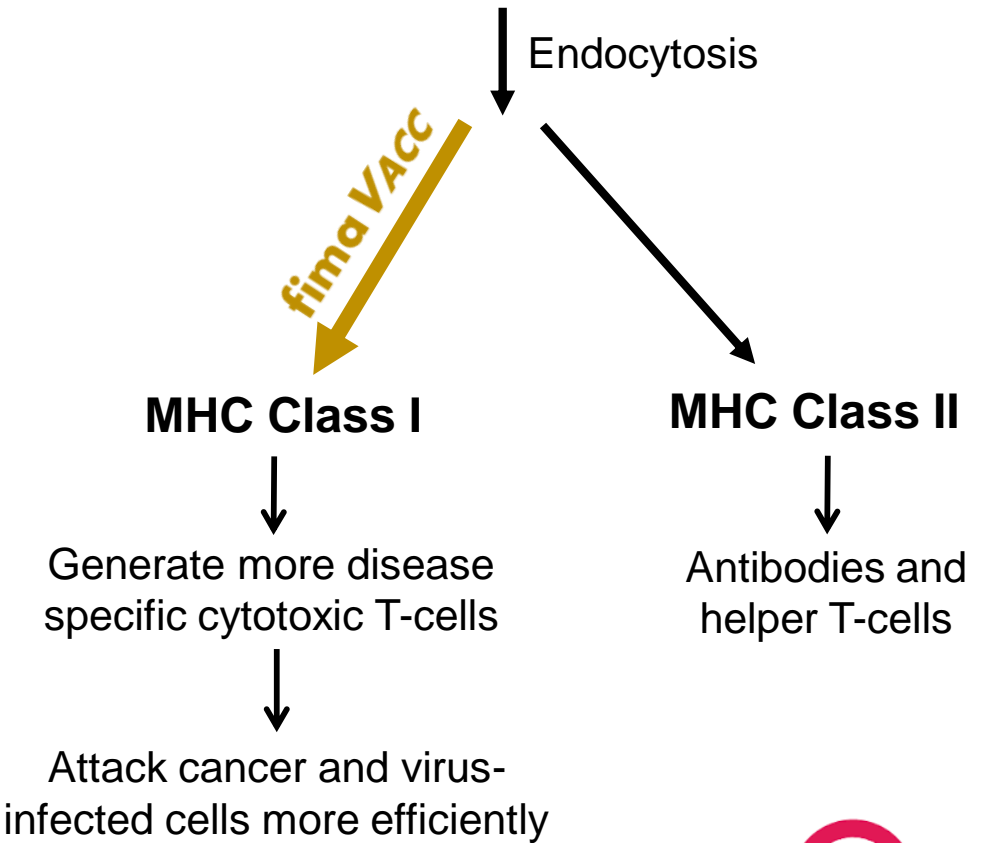
► **fima VACC** – mode of action

## Dendritic cell



● vaccine antigen

## Vaccine



# PROGRESSING CLINICAL TRANSLATION

## ▶ Phase I study in healthy volunteers

---

### ▶ Overall objective:

- Determine the safety, tolerability and immune response of **fima VACC** in healthy subjects

### ▶ Study consists of three parts:

1. Tolerability of intradermal fimaporfin, adjuvant and light (without vaccine)
2. **fima VACC** vaccination: dose finding (fimaporfin and light) and cohort expansion
3. Optimisation of the **fima VACC** regimen

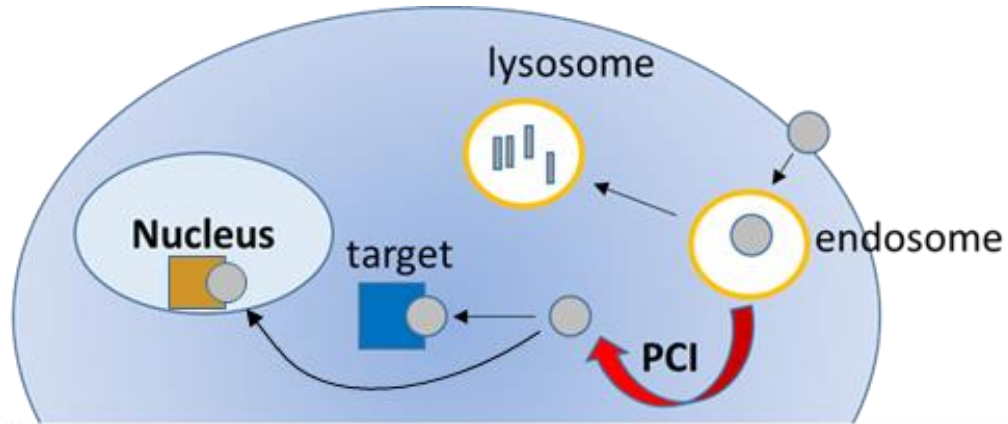
### ▶ Status:

- More than 90 subjects have so far been included
- Part 1 is completed
- Part 2 is completed
  - Initial data suggest overall T-cell enhancement at tolerable doses, as well as early responses and high response rates
  - Vast number of study samples available – near-term focus on characterisation of the immune response
- Part 3 TBD
- Expected study completion: 2H 2018

# PCI TECHNOLOGY

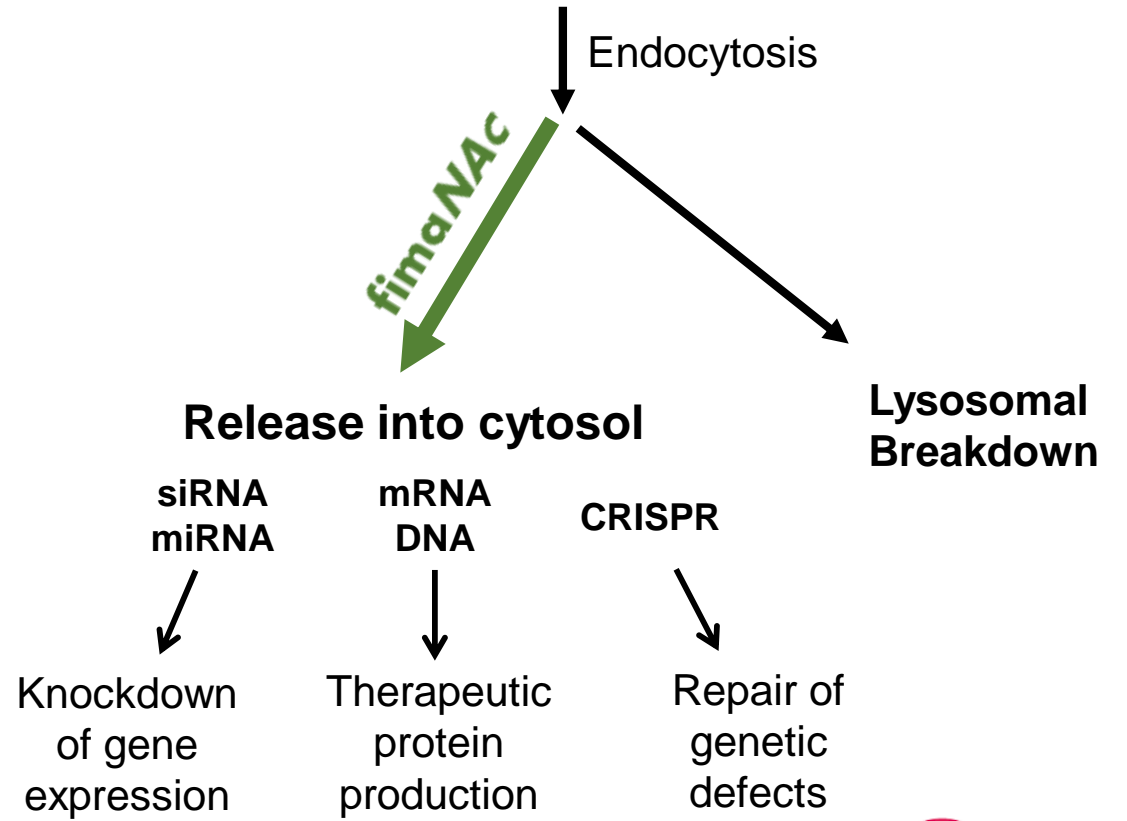
► **fimaNAC** – mode of action

## Target cell



● nucleic acid therapeutic

## Nucleic Acid Therapeutics



# RESEARCH COLLABORATIONS

- ▶ Five active collaborations within nucleic acid therapeutics and vaccination

## fimaNAC



### RXi Pharmaceuticals

- Initiated Q2 2015. Listed on Nasdaq, developing innovative therapeutic siRNA
- Expanded to immuno-oncology following RXi's MirlImmune acquisition

### Top-10 large pharma

- Initiated Q3 2015. A global leader in nucleic acid therapeutics
- Expanded to include *in vivo* studies – current agreement to end of 1H 2018, but may be further extended



### BioNTech

- Initiated Q3 2016. German biotech company developing individualised cancer immunotherapies
- Clinical programmes in melanoma, head & neck, breast, ovarian and pancreatic cancer



### eTheRNA

- Initiated Q4 2016. A global leader in mRNA-based immunotherapies
- Evaluate synergistic effects between companies' technologies

## fimaVACC



### Ultimovacs

- Initiated Q1 2016. Norwegian immunotherapy company
- Therapeutic cancer vaccine against human telomerase

*Research collaborations aim to evaluate synergies between the fima platform and partner technologies, with the potential for further partnerships*

# FINANCE

## ► Key financial figures

| (in NOK 1,000)    | Q1 2018 | Q1 2017 |
|-------------------|---------|---------|
| Other income      | 2,238   | 2,428   |
| Operating results | -14,663 | -9,854  |

| (in NOK 1,000) | Q1 2018 | Q1 2017 |
|----------------|---------|---------|
| Cash           | 38,586  | 69,929  |

| (in NOK 1,000)                 | Q1 2018 | Q1 2017 |
|--------------------------------|---------|---------|
| Cash flow operating activities | -12,236 | -9,327  |

- Public grants in line with last year
- Operating result impacted by increased clinical activity for **fimaCHEM** and **fimaVACC**
- Cash position to cover preparations for **fimaCHEM** pivotal phase
- Oslo Børs listing



# KEY MILESTONES ANTICIPATED

## ▶ Through 2018

---

- 1H 2018 ✓ **Corporate** Transfer of listing from Oslo Axess to Oslo Børs
- 2H 2018 ➤ **fimaCHEM** Safety of repeated treatment
- 2H 2018 ➤ **fimaCHEM** Initiation of pivotal bile duct cancer study
- 2H 2018 ➤ **fimaVACC** Phase I in healthy volunteers completed

# PCI BIOTECH HOLDING ASA

## ► Enquiries

---

CEO Per Walday

Cell phone: +47 917 93 429

Telephone: +47 67 11 54 00

E-mail: [pw@pcibiotech.com](mailto:pw@pcibiotech.com)

CFO Ronny Skuggedal

Cell phone: +47 940 05 757

Telephone: +47 67 11 54 00

E-mail: [rs@pcibiotech.com](mailto:rs@pcibiotech.com)

[www.pcibiotech.com](http://www.pcibiotech.com)