

# PCI BIOTECH

Unlocking the potential of innovative medicines

Q3 2017 PRESENTATION

November 28, 2017

Per Walday, CEO

Ronny Skuggedal, CFO



# PCI BIOTECH

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# HIGHLIGHTS

► Q3 2017

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**fima** *CHEM*

- Granted US Orphan Drug Designation
  - Phase I extension study progressing according to plan
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**fima** *VACC*

- Promising interim clinical results from Phase I suggesting enhancement of several parameters of importance for vaccination
  - Expanding the clinical Phase I study to identify optimal dosing
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**fima** *NAC*

- Extension of the top-10 pharma collaboration
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**Corporate**

- Strengthened management team further by the appointment of Dr Hans Olivecrona as Chief Medical Officer

# PCI BIOTECH AT A GLANCE

## ▶ Unlocking the potential of innovative medicines

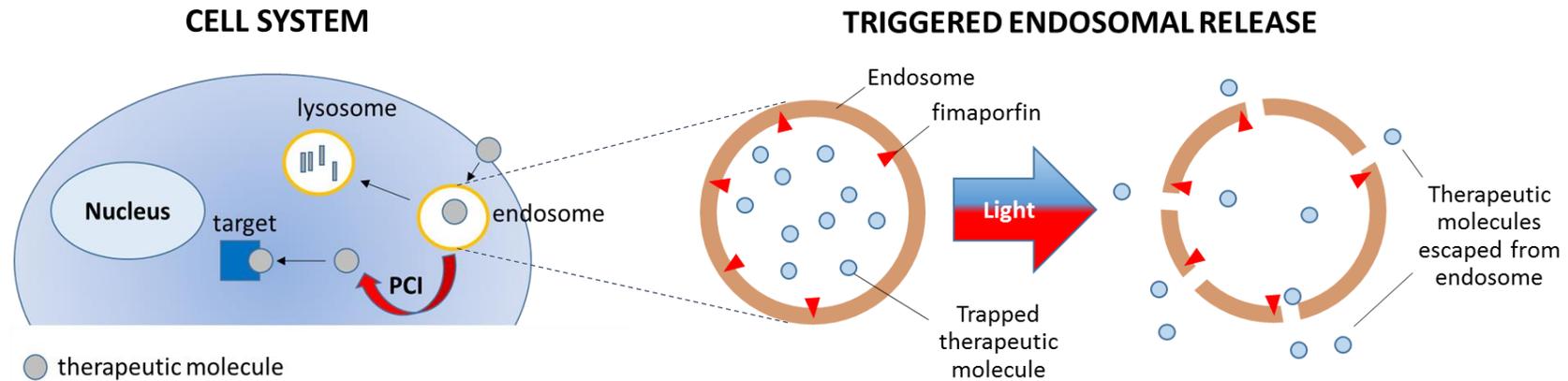
- ▶ A listed (PCIB:NO) cancer-focused biotech company
- ▶ Photochemical internalisation (“PCI”) technology, originating from the Norwegian Radium Hospital

Programme	Indications / Therapeutics	Preclinical	Phase I	Phase II	Status
	 <i>Bile duct cancer / gemcitabine</i>				Phase I extension to evaluate safety of repeated treatment in the orphan indication bile duct cancer
	 <i>Therapeutic cancer vaccines</i>				Phase I study in healthy volunteers One active R&D collaboration
	 <i>Nucleic acid therapeutics</i>				Four active R&D collaborations

*An oncology focused company with three well differentiated assets*

# PCI TECHNOLOGY

► Enabling drugs to reach intracellular therapeutic targets



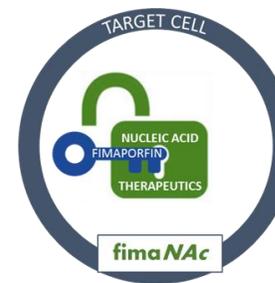
***PCI – the solution to a key challenge for several modalities***



Enabling approved drugs to fulfil unmet local treatment need



Enhancing cellular immune responses important for therapeutic effect

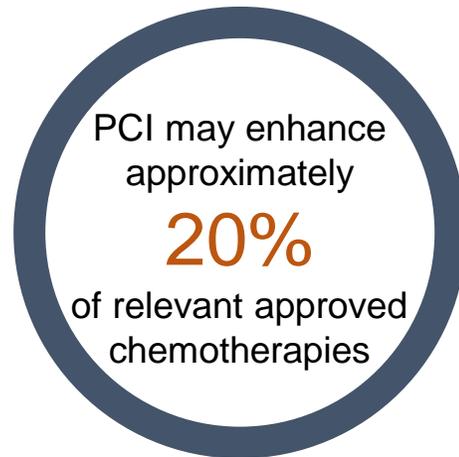


Providing a delivery solution for nucleic acid therapeutics

# THE SOLUTION TO A KEY CHALLENGE

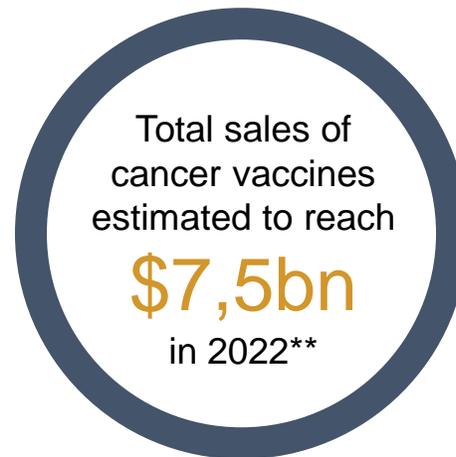
## ▶ Three well-defined development programmes

### 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 disease 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

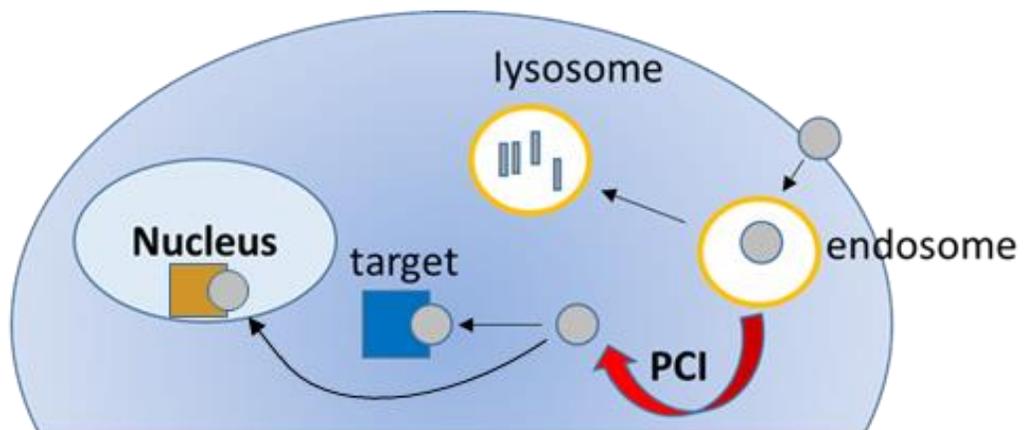
\*\* GBI Research (2016) Global Cancer Vaccines Market to 2022

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

# PCI TECHNOLOGY

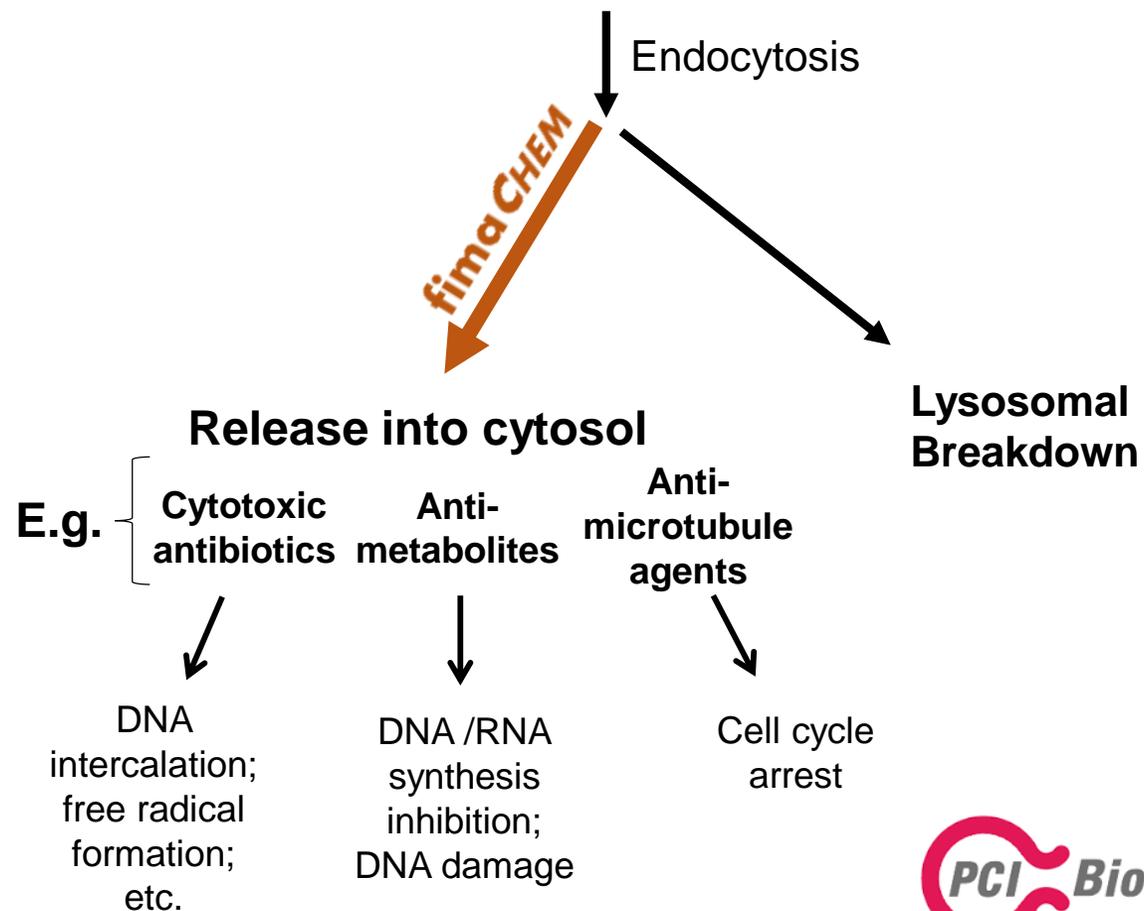
► fimaCHEM – mode of action

## 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**
- Conjoining localised with systemic therapy

### 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

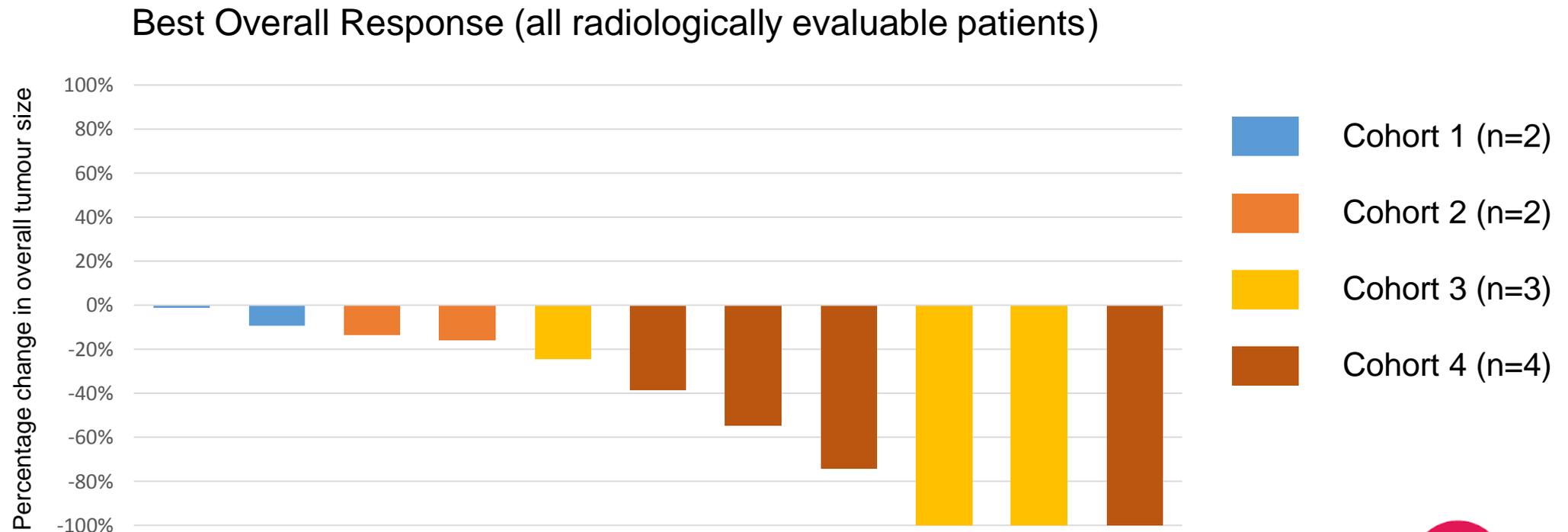
### Inducing immunogenic tumour cell death

- 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

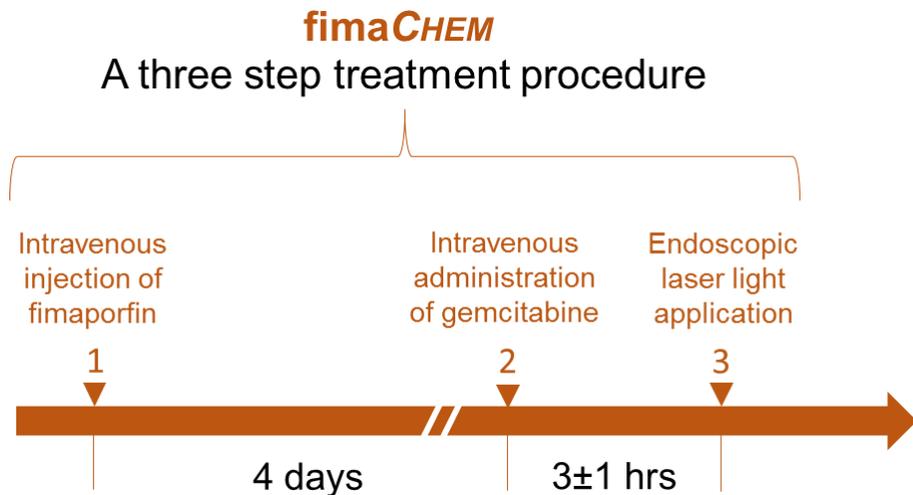
## ► Encouraging early signs of efficacy in Phase I

- Interim average overall survival (OS) of all 16 patients in Phase I was 16.5 months per November 2017, with 25% of the patients still being alive. Median OS ended at 14.4 months.

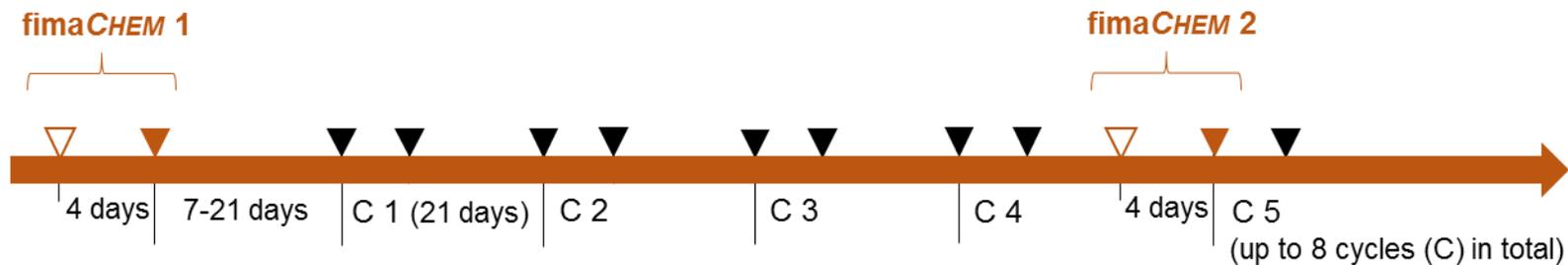


# BILE DUCT CANCER – PHASE I EXTENSION STUDY

▶ 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



- ▽ fimaporfin
- ▼ gemcitabine + light
- ▼ gemcitabine + cisplatin

# INOPERABLE EXTRAHEPATIC BILE DUCT CANCER

## ▶ Status and strategy going forward

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### ▶ **Orphan designation**

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

### ▶ **Phase I completed with good tolerability and promising early signs of efficacy**

- Tumour shrinkage in almost all radiologically evaluable patients
- Interim average overall survival (OS) of 16.5 months with 25% of patients still alive; median OS ended at 14.4 months

### ▶ **Exploring safety of repeated treatment as a Phase I extension**

- First patient included in August 2017 – study progressing according to plan

### ▶ **Regulatory interactions with authorities to determine fastest way to market**

- Interactions continued during Q3 with productive discussions to establish a strong and viable development strategy

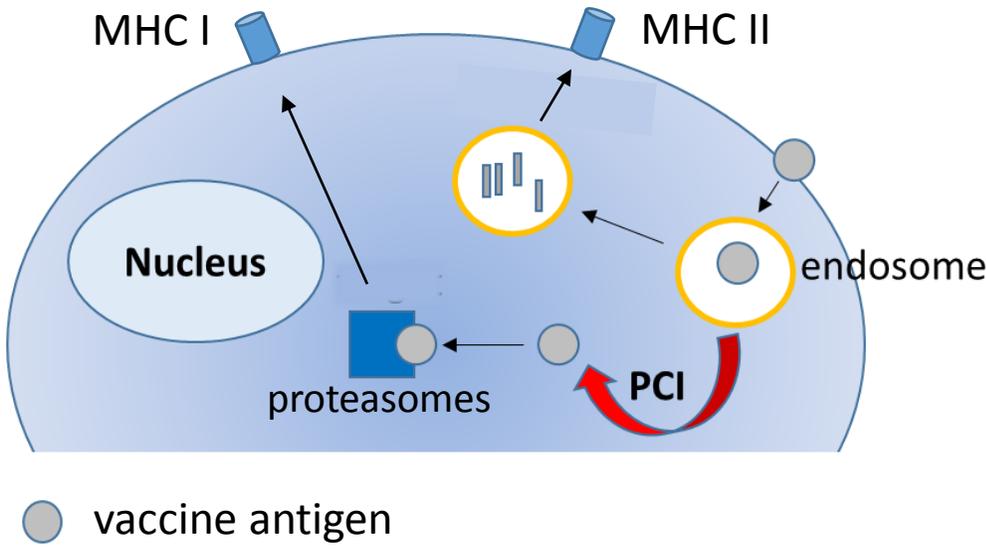
### ▶ **Engaging US key opinion leaders (KOL's)**

- Engaging US KOL's and conducted a clinical advisory board meeting to advise on Phase II design

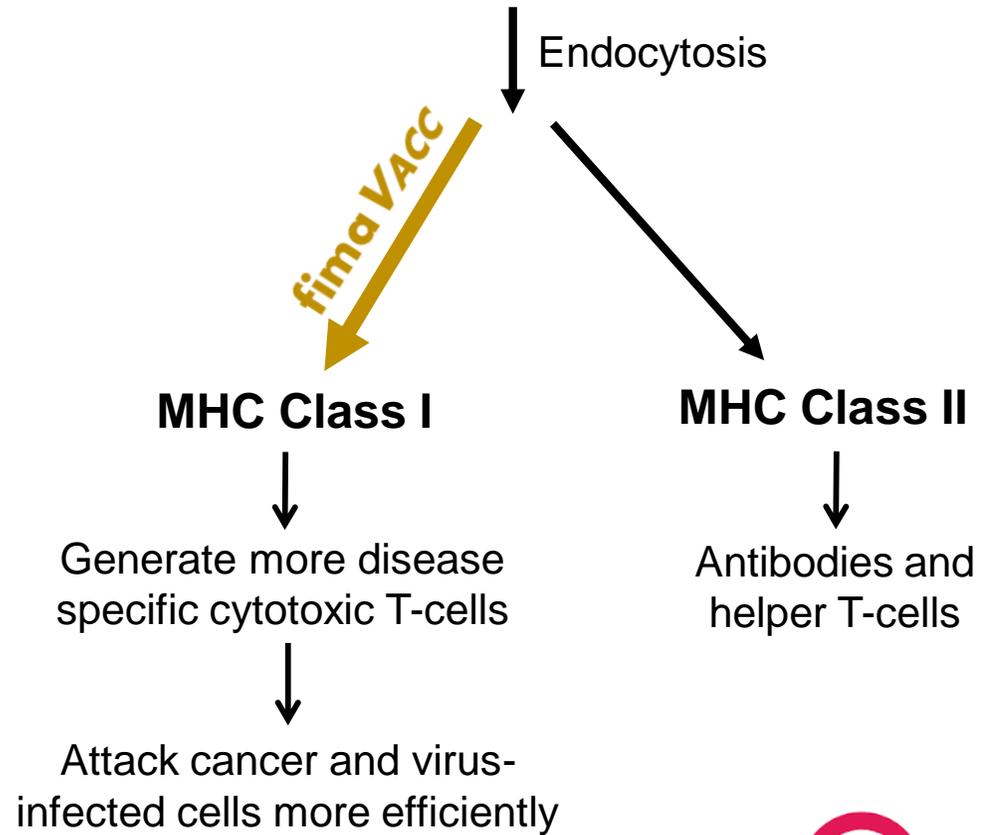
# PCI TECHNOLOGY

► **fima VACC** – mode of action

## Dendritic cell



## Vaccine



# PROGRESSING CLINICAL TRANSLATION

## ▶ Phase I study in healthy volunteers

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### ▶ 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

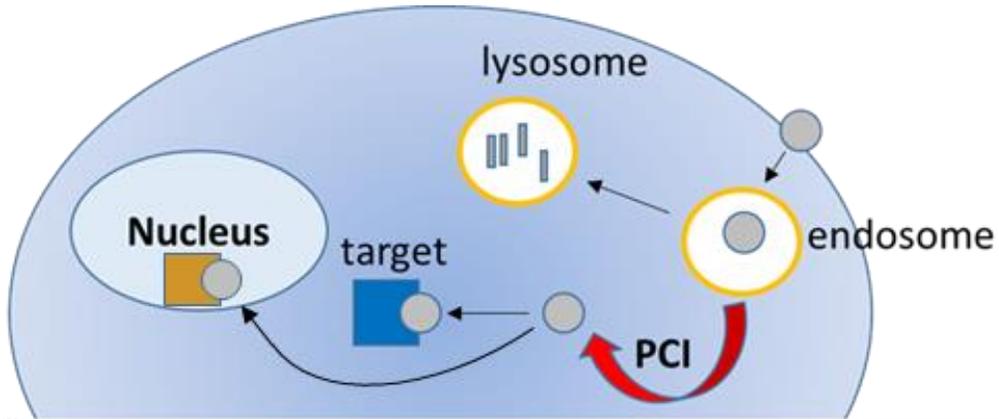
### ▶ Status:

- More than 70 subjects have so far been included
- Part 1 is completed
- Part 2 is ongoing
  - Initial data suggest overall T-cell enhancement at tolerable doses, as well as early responses and high response rates
  - Notably, best responses at the lowest fimaporfin dose tested → study will be expanded to include lower doses
- Part 3 not yet started
  - Optimisation of timing of light
  - Expected completion: 2H 2018

# PCI TECHNOLOGY

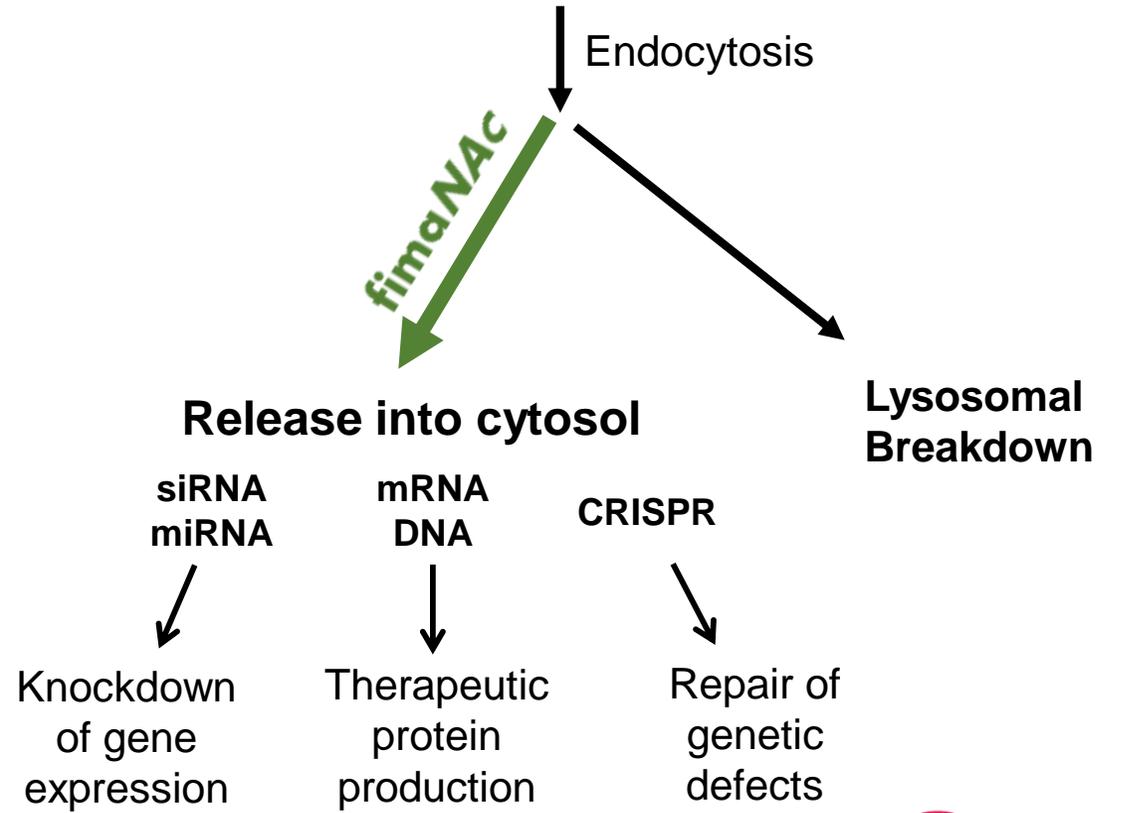
► **fimaNAC** – mode of action

## 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 MirImmune 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 2017, 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

<i>(In NOK 1,000)</i>	2017 Q3	2016 Q3	2017 YTD	2016 YTD
<b>Operating results</b>	<b>-10 456</b>	<b>-9 535</b>	<b>-27 507</b>	<b>-26 124</b>
<b>Cash &amp; cash equivalents</b>	<b>53 755</b>	<b>20 663</b>	<b>53 755</b>	<b>20 663</b>

- ▶ SkatteFUNN grant of NOK 5.9 million to be received in Q4
- ▶ Cash position to reach key milestones:
  - Initiation of **fimaCHEM** Phase II
  - Completion of **fimaVacc** Phase I

# GOOD PROGRESS IN ALL AREAS

▶ YTD 2017

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- ✓ **fimaCHEM** Granted orphan designation in the US
- ✓ **fimaCHEM** Initiated patient enrolment in the extension of Phase I
- ✓ **fimaVACC** Tolerability of the vaccination technology established
- ✓ **fimaVACC** Promising initial immune response results
- ✓ **fimaNAC** Preclinical research collaborations entering new stages
- ✓ **Finance** Secured financing to reach key milestones
- ✓ **Corporate** Strengthened the organisation with Dr Olivecrona as CMO

# KEY MILESTONES ANTICIPATED

## ▶ Through 2018

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- 2H 2017   ▶ **fimaCHEM**   Regulatory clarity on fastest way to market
- 1H 2018   ▶ **fimaCHEM**   Safety read-out of Phase I extension
- 1H 2018   ▶ **fimaCHEM**   Initiation of Phase II
- 2H 2018   ▶ **fimaVACC**   Phase I in healthy volunteers completed

# PCI BIOTECH

## ▶ Summary and outlook

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### ▶ **fimaCHEM**

- Granted orphan drug designation in the US
- Strong tumour response and encouraging survival data
- Phase I extension study progressing according to plan
- Productive regulatory interactions approaching outcome
- Engaged US KOL's and discussed clinical design, preparing for Phase II

### ▶ **fimaVACC**

- Tolerability established
- Promising initial results with several features of importance for vaccination platforms
- Notably, best responses at lowest dose – expanding dose finding to explore lower doses

### ▶ **fimaNAc**

- Top-10 pharma collaboration entered *in vivo* studies and extended until end of 2017 – may be further extended

### ▶ Cash position to reach key milestones

# PCI BIOTECH HOLDING ASA

## ► Enquiries

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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)