



# PCI BIOTECH

Unlocking the potential of innovative medicines

## Q4 & Interim Full Year 2018 PRESENTATION

February 13, 2019

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

► Fourth quarter 2018

---

## **fima** *CHEM*

- Preparations for the RELEASE study progressing towards initiation in 1H 2019
- Continued positive early signs of efficacy from Phase I dose-escalation
- Preliminary confirmation of safety read-out from the Phase I extension study
- Presented Phase I dose-escalation results at 2018 ESMO congress and at the annual conference of the US CCA Foundation in Jan 2019 (subsequent event)

# HIGHLIGHTS

► Fourth quarter 2018

---

## **fima VACC**

- Phase I interim data suggest enhancement of several parameters of importance for vaccination
- US patent granted for “band-aid-like” device for skin illumination/injection

# HIGHLIGHTS

► Fourth quarter 2018

---

## **fima***NAC*

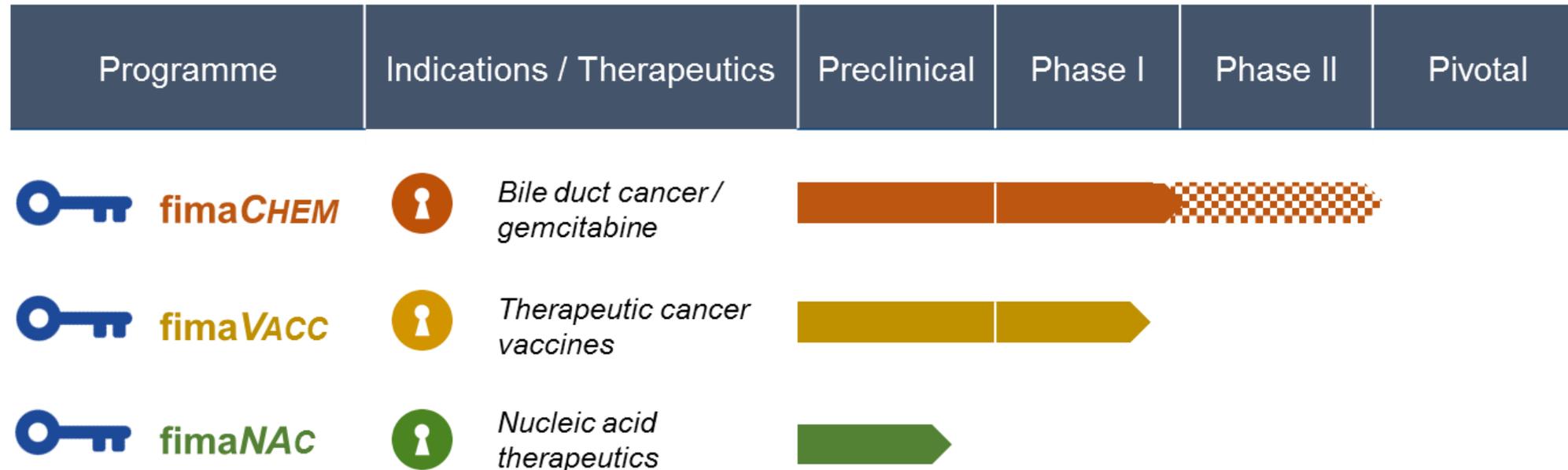
- Extension of preclinical research collaboration agreement with a top-10 large pharma company

## **Corporate**

- Completed fully underwritten rights issue of NOK 360 million
- Further strengthened the clinical organisation and the Scientific Advisory Committee

# 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

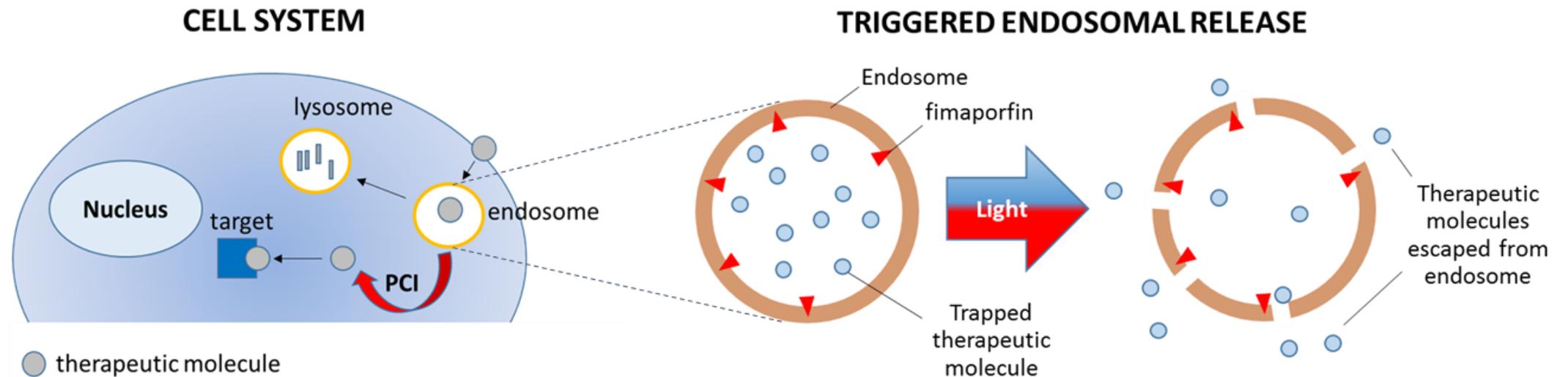


*An oncology focused company with three well differentiated assets*

# PCI TECHNOLOGY

- ▶ Enabling drugs to reach intracellular therapeutic targets

## *Mode of action*



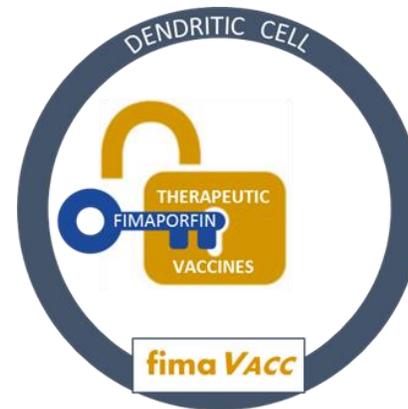
# PCI TECHNOLOGY

- ▶ Enabling drugs to reach intracellular therapeutic targets

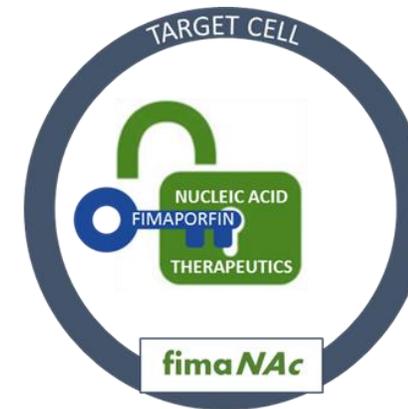
## *PCI – the solution to a key challenge for several modalities*



Enabling approved drugs to fulfil unmet local treatment need



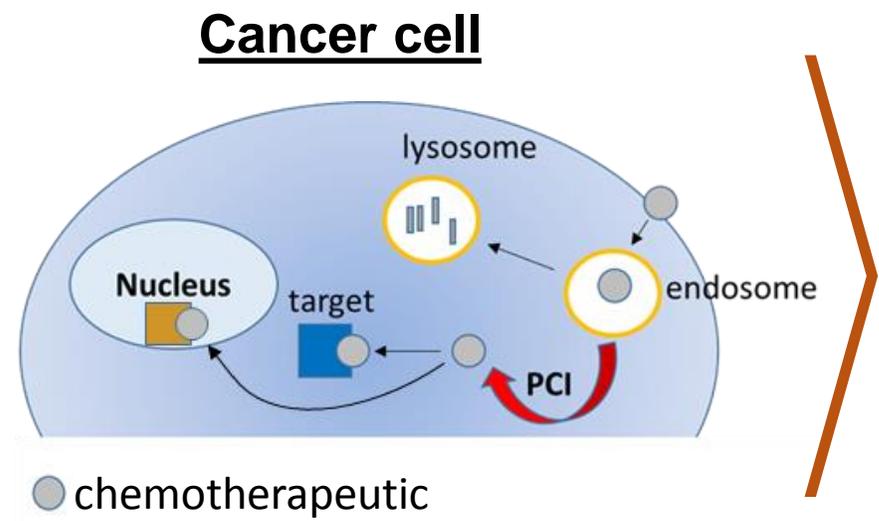
Enhancing cellular immune responses important for therapeutic effect



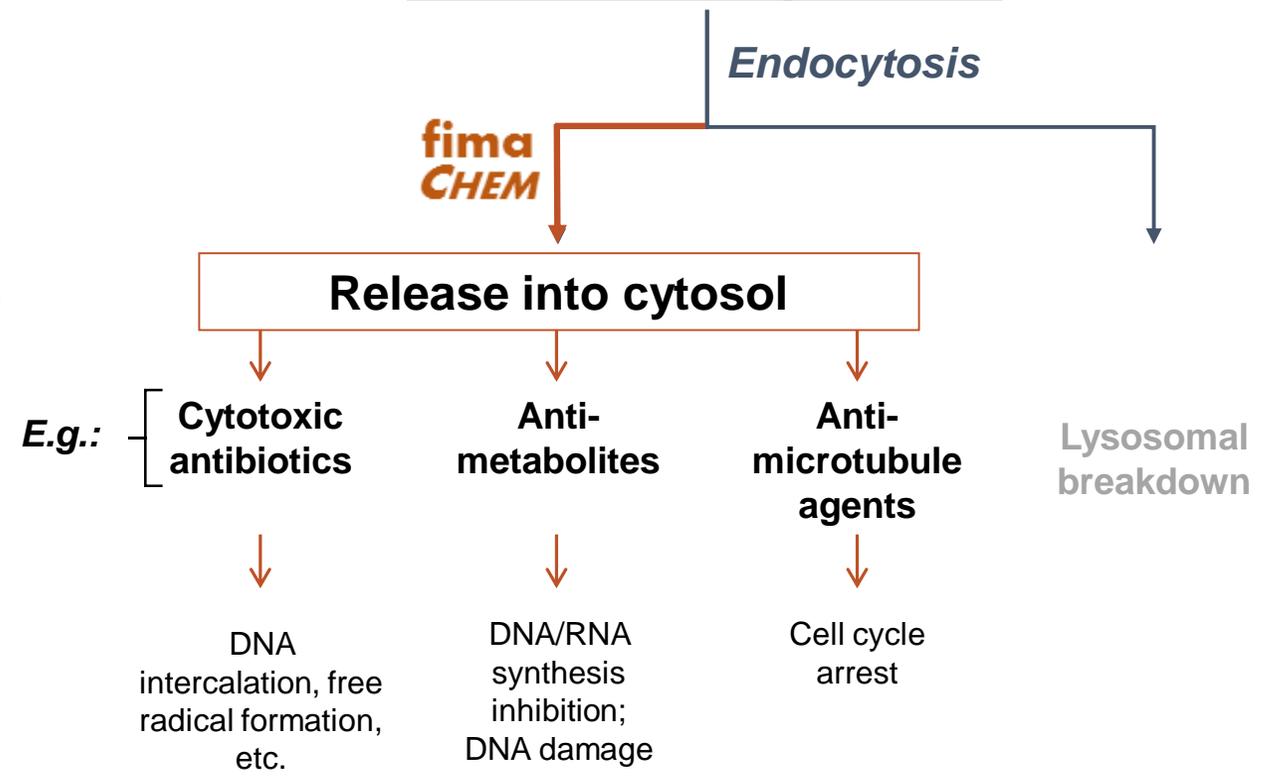
Providing a delivery solution for nucleic acid therapeutics

# PCI TECHNOLOGY

► **fimaCHEM** – mode of action



## Chemotherapeutics



# BILE DUCT CANCER – EXTRAHEPATIC INOPERABLE

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

- ▶ Orphan indication
- ▶ Incidence: 1-2/100,000 in western world – higher in Asia
- ▶ Average survival inoperable: 11-12 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

Easy illumination  
through standard  
endoscopic methods

Boosting chemotherapy  
effect where it is most  
needed

# BILE DUCT CANCER – CLINICAL PHASE I STUDY

► Cohort IV is selected dose for pivotal study – limited but encouraging data (Jan 2019)

**Continued positive early signs of efficacy – mOS determined to 21.7 months at selected dose**

Parameters	Cohort IV (N=6) (0.25mg/kg)	Phase I – full study (N=16) (0.06-0.25mg/kg)
<b>Objective Response Rate (ORR)</b>	<b>3/5 patients</b> (2 PR; 1 CR)	<b>4/12 patients</b> (2 PR; 2 CR)
<b>Median Overall Survival (mOS)</b>	<b>21.7 months</b>	<b>14.4 months</b>

# BILE DUCT CANCER – RELEASE STUDY

## ▶ Progress towards initiation of the pivotal study

---

### ▶ Preparations progressing towards initiation of RELEASE in first half 2019

- Preliminary confirmation of achieved safety endpoint in the extension study – final confirmation pending completion of site monitoring and formal review by the appointed Cohort Review Committee
- Ongoing regulatory, ethics and contract negotiations progressing well – all country approvals in Norway
- Strengthened the clinical organisation by appointing Karin Staudacher, M.Sc. as Clinical Project Director
- Presentation of Phase I data at the 2018 ESMO and the US CCA Foundation conference (Jan'19)

### ▶ Orphan designation

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

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

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

# BILE DUCT CANCER – RELEASE STUDY

## ► Randomised study with interim analysis for potential accelerated/conditional approval

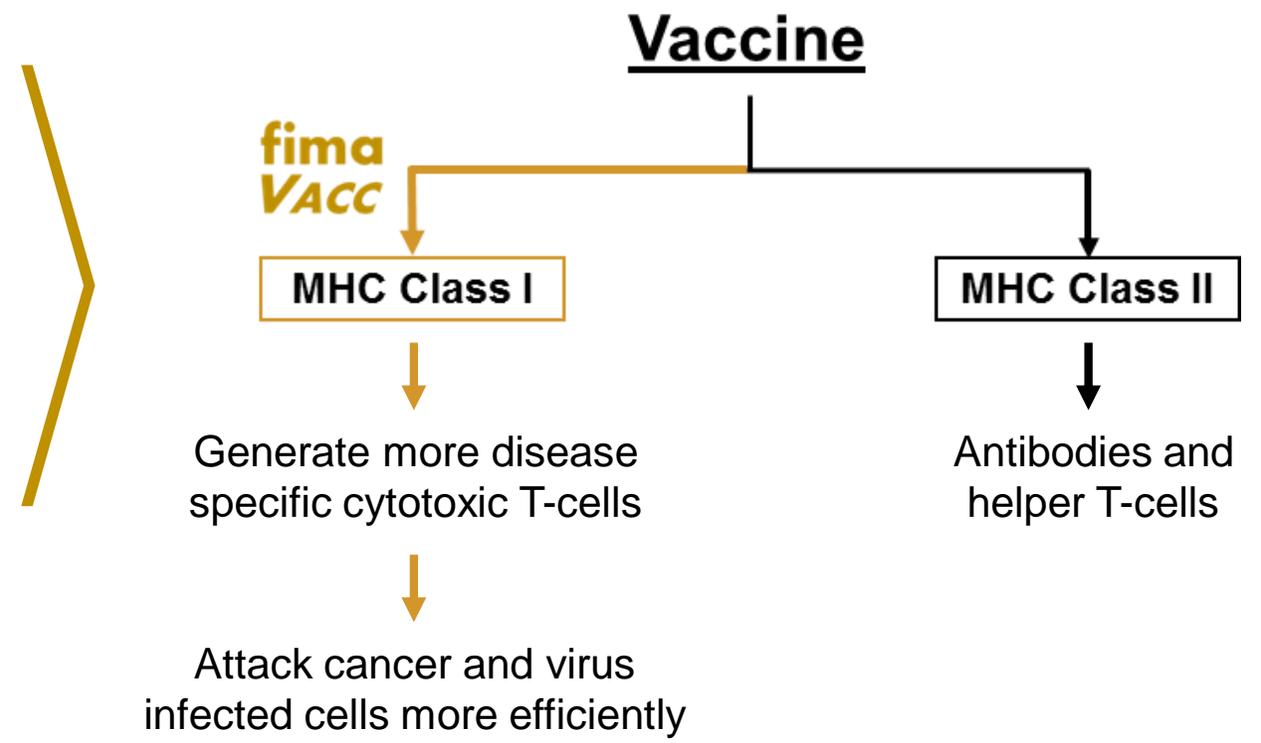
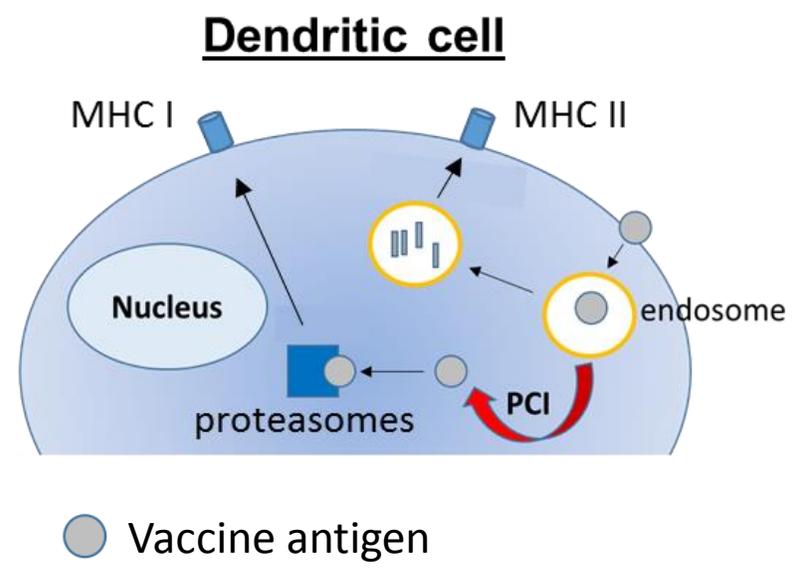
- First line treatment of patients with inoperable extrahepatic bile duct cancer
- Approx. 40 key hospitals (Europe & USA)
- Approx. 36 months to interim and 50 to final analysis
- Randomisation (1:1) of 186 patients
- Primary endpoint: PFS<sup>a</sup>, with OS<sup>b</sup> as key secondary
- Interim analysis primary endpoints: PFS followed by ORR<sup>c</sup>

### **RELEASE trial progress reporting:**

- Key milestones will be communicated in press releases
  - Start of study (first patient dosed); IDMC<sup>d</sup> recommendations; clinical results presentations, filing, etc.
- Progress will be updated in quarterly reports
  - Number of country approvals
  - Number of sites open for enrolment

# PCI TECHNOLOGY

▶ **fima VACC** – aiming to enhance immunogenicity of vaccines for immunotherapy field



# PROGRESSING CLINICAL TRANSLATION

▶ Phase I study in healthy volunteers

---

▶ Overall objective:

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

▶ Study consists of three parts:

1. Tolerability – *completed*
2. **fima VACC** vaccination – *completed*
3. Optimisation of the **fima VACC** regimen – *tbd*

# PROGRESSING CLINICAL TRANSLATION

▶ Phase I study in healthy volunteers

---

▶ Status:

- More than 90 subjects treated
- Data generated so far suggest enhancement of antigen specific T-cell response at tolerable doses:
  - ✓ earlier responses
  - ✓ higher response rates

# PROGRESSING CLINICAL TRANSLATION

## ▶ Phase I study in healthy volunteers

---

### ▶ Status:

- In-depth analyses of T-cell responses by Prof. van der Burg's group
  - High bar set for induction of CD8 T-cells – notoriously difficult to induce responses in man with selected vaccine antigen
  - Data from the first two **fima VACC** cohorts analysed suggest that the vaccination regimen provides a higher number of responders (5 of 6) compared to control (2 of 6)
  - Analyses of further cohorts will be completed with the aim of confirming these preliminary positive findings prior to publication

## SAC REINFORCEMENT AND PATENT GRANTED

- ▶ SAC reinforced with immunological expertise and device patent granted
- ▶ Immunological expertise in the Scientific Advisory Committee (SAC) further strengthened by the addition of Professor Sjoerd van der Burg
- ▶ Device patent granted in the US in Oct 2018

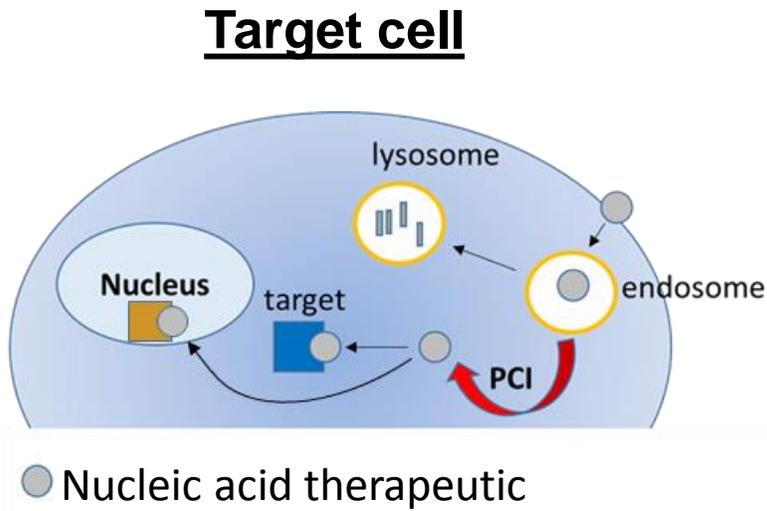


*Disposable “band-aid-like” device for user-friendly illumination/injection of the vaccination site*

- ▶ Collaboration with Ultimovacs ended based on strategic considerations

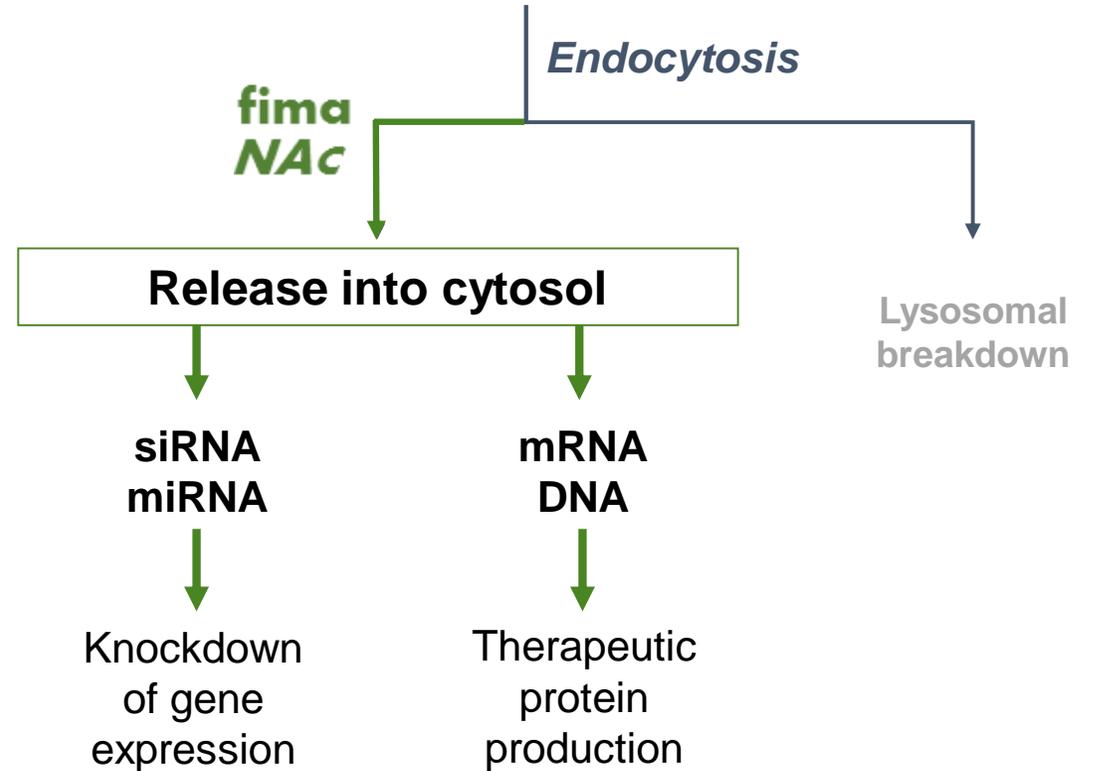
# PCI TECHNOLOGY

## ► **fimaNAC** – mode of action



*E.g.:* {

## Nucleic acid therapeutic



# RESEARCH COLLABORATIONS

▶ Six collaborations established with key players in nucleic acid therapeutics

- ▶ Two new research collaborations established in 2018
- ▶ Top-10 large Pharma collaboration extended to end of 1H'19

fimaNAC

Top-10  
large  
pharma



\* Previously named RXi Pharmaceuticals

# FINANCE

▶ Fully underwritten rights issue of NOK 360 million completed in October 2018

---

- ▶ Important milestone for the **fimaCHEM** development programme
  - expected to fund the RELEASE study beyond interim read-out

▶ The rights issue

- supported by major shareholders and 87% subscription
- net proceeds of NOK 328 million
- part of proceeds placed in Euro to hedge the project currency risk

# FINANCE

## ▶ Key financial figures

- ▶ Other income (public grants) in line with previous year
- ▶ Operating result impacted by R&D activity for **fimaCHEM** and **fimaVacc**

(in NOK 1,000)	Q4 2018	Q4 2017	FY 2018	FY 2017
Other income	2,972	3,067	9,585	10,250
Operating results	-14,278	-15,924	-44,519	-43,431

(in NOK 1,000)	Q4 2018	Q4 2017	FY 2018	FY 2017
Net cash flow operating activities	0*	-2,966	-30,297	-29,943

(in NOK 1,000)	31.12 2018	31.12 2017
Cash	349,326	50,789

\* NOK 9 million positive effect from Euro bank deposits for Q4 2018

# KEY ACHIEVEMENTS & NEAR-TERM MILESTONES

---

- 2H 2018 ✓ **Corporate** Financing for pivotal **fimaCHEM** study
- 2H 2018 ✓ **fimaNAc** Established two new research collaborations
- 2H 2018 ✓ **fimaCHEM** Design of pivotal study finalised
- 2H 2018 (✓) **fimaCHEM** Safety of repeated treatment
- 1H 2019 ➤ **fimaCHEM** Initiation of pivotal bile duct cancer study
- 1H 2019 ➤ **fimaVacc** Completion of Phase I immune analyses (timeline adjusted from 2H 2018)

# INVESTMENT HIGHLIGHTS

## Market

**Platform technology with three programmes** targeting an **attractive and growing oncology market**, with a clear path to a **high unmet need orphan oncology market** for the lead product candidate

## Lead product

**Amphinex®** is a **pivotal phase ready orphan designated (EU & US) first-in-class** photochemical internalisation product for treatment of bile duct cancer – a **disease without approved drugs**

## Clinical results

**Positive early signs of tumour response** in a first-in-man study published in Lancet Oncology, and in a Phase I study specifically targeting bile duct cancer – **encouraging survival data**

## Pipeline

**fima VACC** – a clinical stage vaccination technology with **encouraging cellular immune responses**  
**fima NAc** – a preclinical gene therapy delivery solution with **established key player collaborations**

## Strategy

Development strategy for **lead candidate** established based on **thorough regulatory discussions** with FDA and EMA – a single randomised pivotal study with **accelerated/conditional approval potential**

## Leadership

Management team, Board of Directors and advisors with **extensive pharmaceutical industry experience** across a range of medical development and commercial areas

## FOR ENQUIRIES

**Per Walday, CEO**

Mobile phone: +47 917 93 429

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

**Ronny Skuggedal, CFO**

Mobile phone: +47 940 05 757

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