



# PCI BIOTECH

Unlocking the potential of innovative medicines

## Q3 2019 PRESENTATION

November 27, 2019

Per Walday, CEO

Ronny Skuggedal, CFO



# PCI BIOTECH










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# PCI BIOTECH – UNLOCKING THE POTENTIAL OF INNOVATIVE MEDICINES

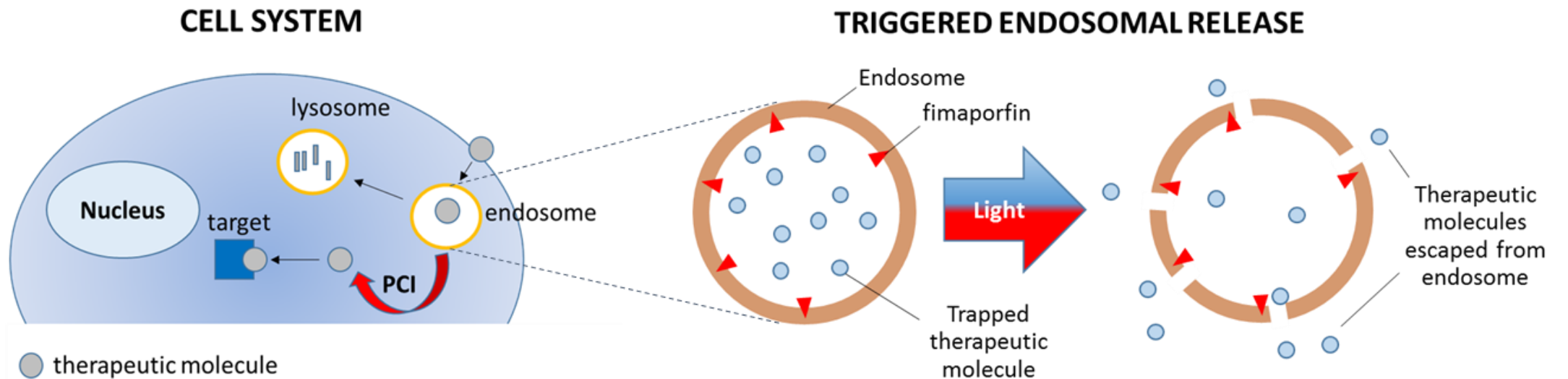
An oncology company with three well differentiated assets

Programme	Indications/Therapeutics	Preclinical	Phase I	Phase II	Pivotal
 <b>fimaCHEM</b>	 <i>Bile duct cancer/ gemcitabine</i>				
 <b>fimaVACC</b>	 <i>Therapeutic cancer vaccines</i>				
 <b>fimaNAC</b>	 <i>Nucleic acid therapeutics</i>				

Photochemical internalisation (PCI) is a platform technology with three programmes targeting an attractive and growing oncology market

# PCI TECHNOLOGY – MODE OF ACTION

- ▶ Enabling drugs to reach intracellular therapeutic targets



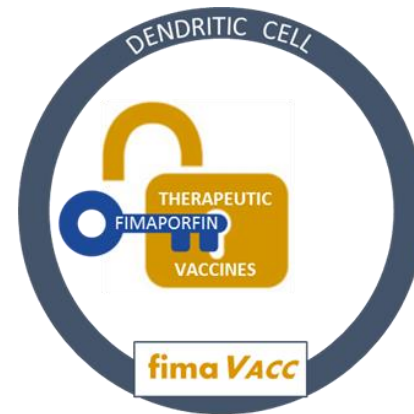
# PCI TECHNOLOGY

- ▶ Enabling drugs to reach intracellular therapeutic targets

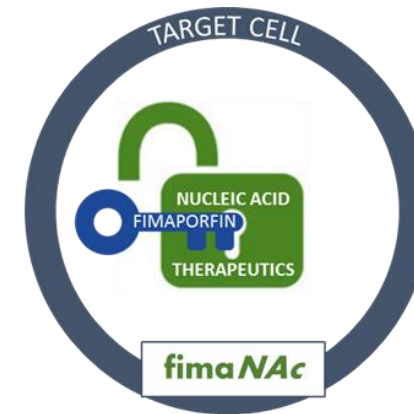
***PCI – the solution to a key challenge for several modalities***



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

# Q3 HIGHLIGHTS

## ▶ **fima** *CHEM*

### **RELEASE – maintaining main milestones**

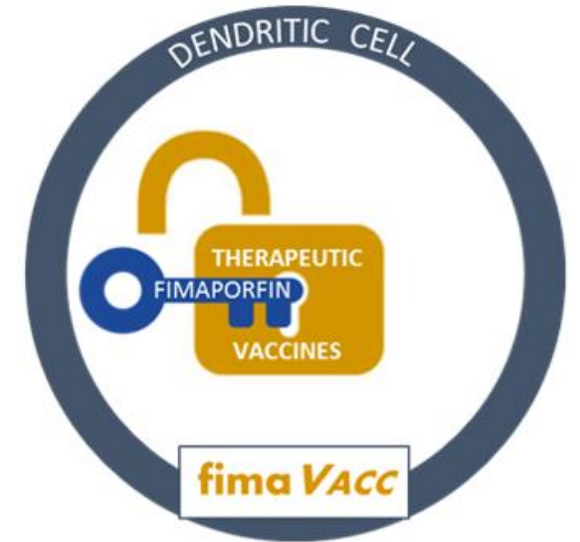
- ▶ First patient enrolled in May 2019
- ▶ Regulatory and ethics approval in 10 of 11 EU countries + USA
- ▶ Opened 23 of total planned 40 sites
- ▶ First US clinical site opened only recently, enrolment of first US patient likely to slide into 2020
- ▶ ~10 sites behind schedule, expecting to catch up in early 2020
- ▶ Site selection ongoing in Asia with the aim to include sites in 2020



# Q3 HIGHLIGHTS

## ▶ **fima VACC**

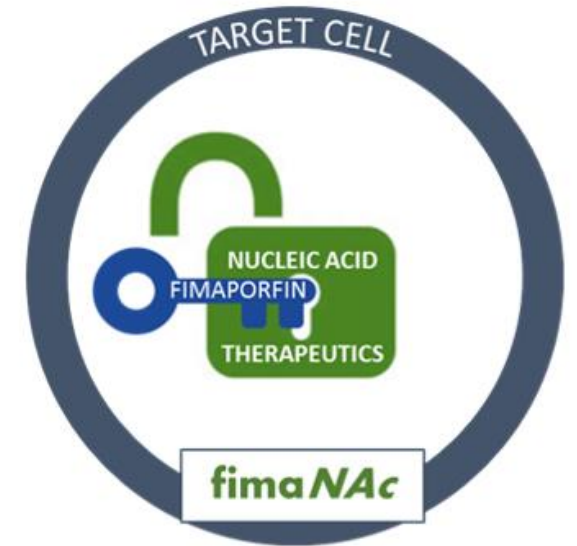
- ▶ Phase I results accepted for presentation at the ESMO Immuno-Oncology Congress
- ▶ Proof-of-Concept of the **fima VACC** technology established with enhanced immune responses in healthy volunteers
- ▶ Publication of preclinical results in 'Frontiers of Immunology', a high-impact journal
- ▶ Parallel development strategies with direct partnering efforts and planning for clinical PoC in disease setting



# Q3 HIGHLIGHTS

## ▶ **fimaNAc**

- ▶ Extended and expanded the AstraZeneca research collaboration through 2019
- ▶ Scope recently expanded to evaluate if synergies in oncology are transferrable to other disease areas
- ▶ Further potential partnership to be evaluated during 1H 2020
- ▶ Promising response on patent application for mRNA delivery, which is highly relevant for several collaboration partners

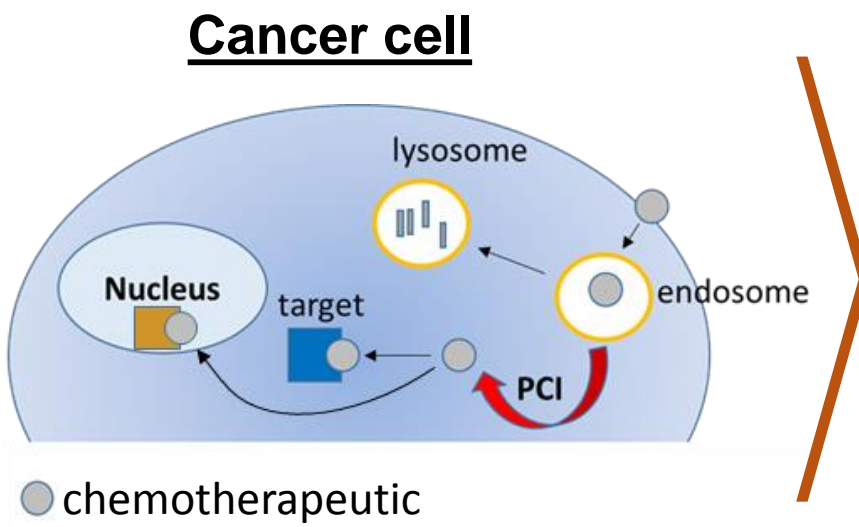




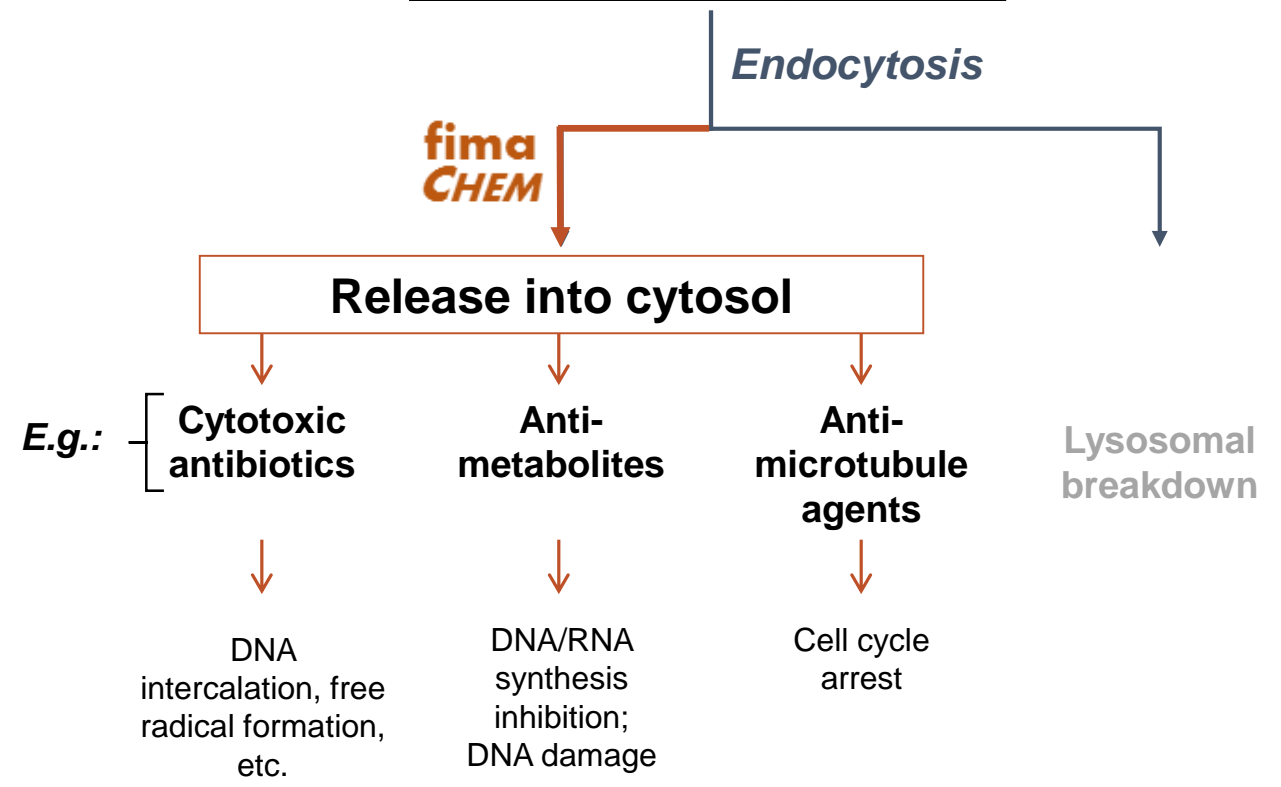


# PCI TECHNOLOGY

► fimaCHEM – mode of action



## Chemotherapeutics



# BILE DUCT CANCER – EXTRAHEPATIC INOPERABLE

▶ **fimaCHEM** – an excellent fit with medical need and existing treatments

## High unmet medical need

- ▶ Only **11-12 months**<sup>1</sup> average survival for inoperable tumors
- ▶ Less than 1/3 of tumors are resectable at presentation
- ▶ No approved chemotherapies; gemcitabine and cisplatin are actively used and recommended
- ▶ Endoscopic stenting for palliative biliary drainage

## **fimaCHEM** advantages

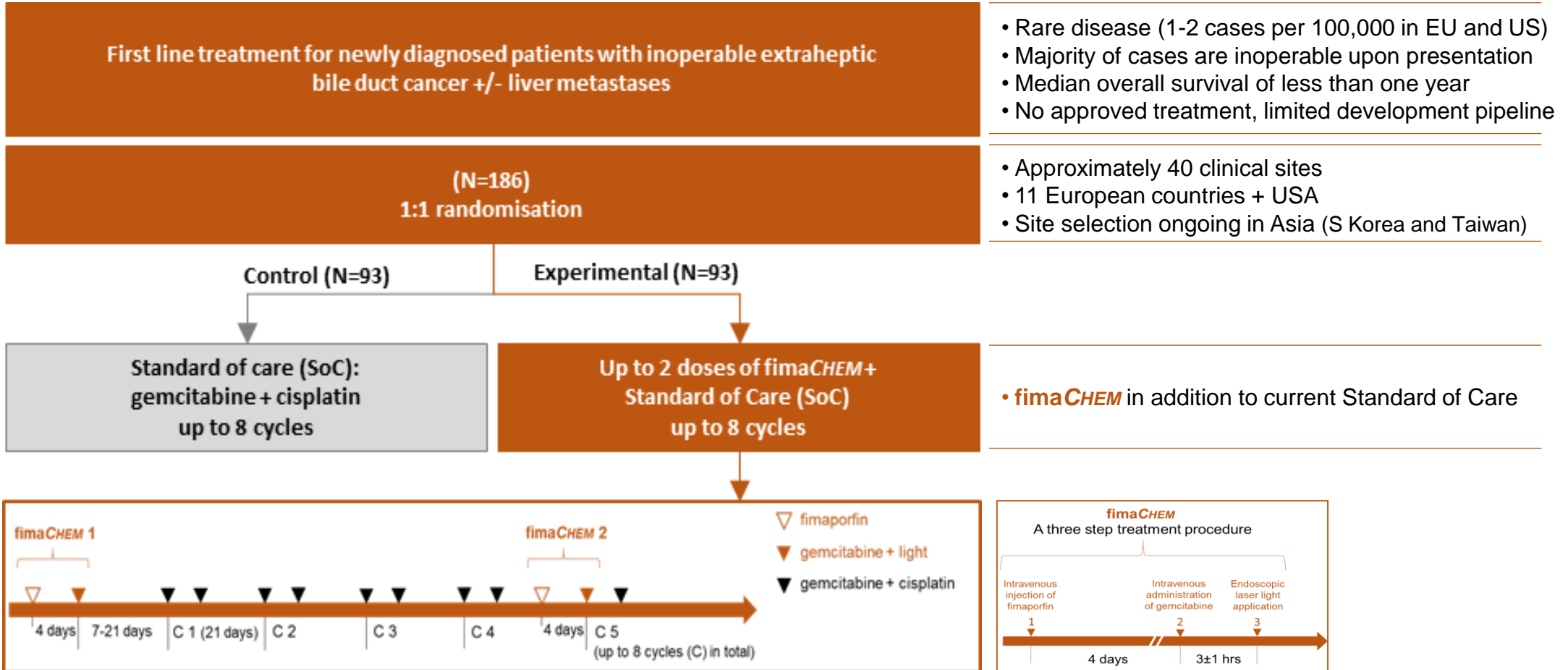
- ▶ mOS<sup>2</sup> of **21.7 months** at selected dose (cohort IV) in Phase I dose-escalation
- ▶ Potentially offers clear benefit for majority of target patient cases
- ▶ Enhances recommended first-line chemotherapy and boosts effect where it is most needed
- ▶ Easy illumination through standard endoscopic methods

<sup>1</sup> New England Journal of Medicine 2010;362:1273-81

<sup>2</sup> Median overall survival

# BILE DUCT CANCER – RELEASE STUDY

► Pivotal study with potential accelerated/conditional approval on interim analysis



# BILE DUCT CANCER – RELEASE STUDY

- ▶ Pivotal study progressing as planned
- ▶ Regulatory and ethics approvals progressing according to plan – approvals received for USA and 10 of 11 planned European countries by mid-November
- ▶ 23 of planned 40 sites open for patient enrolment
- ▶ First US clinic opened only recently, with first US patient likely to slide into 2020
- ▶ Current lag in opening of sites does not affect the main milestones
- ▶ Site selection ongoing for start-up in 2020 in Asia, where bile duct cancer is more prevalent



# BILE DUCT CANCER – RELEASE STUDY

## ► Endpoints, milestones and timelines

### Endpoints:

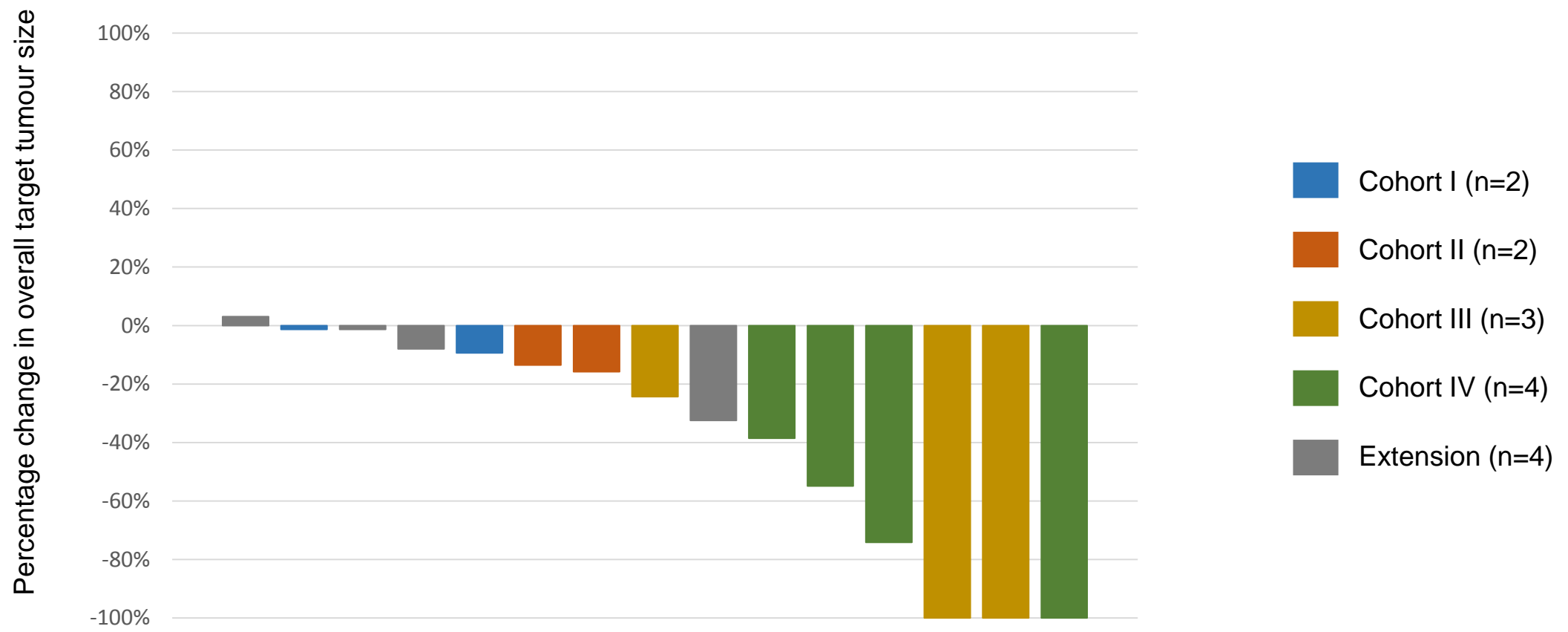
<p><b>Interim analysis:</b> Primary Endpoint: Progression free survival (PFS) Secondary endpoint: Objective Response Rate (ORR)</p>	<ul style="list-style-type: none"> <li>• Orphan drug designation in Europe and USA</li> <li>• Potentially accelerated/conditional approval</li> </ul>
<p><b>Final analysis:</b> Primary endpoint: Progression free survival (PFS) Secondary endpoint: Objective response rate (ORR)</p>	<ul style="list-style-type: none"> <li>• Single randomised trial considered sufficient based on interaction with US and EU regulatory authorities</li> </ul>

### Milestones and timelines:

<p>First patient enrolled in Europe in May 2019</p>	<ul style="list-style-type: none"> <li>• First patient in the US may slide into 1H 2020</li> </ul>
<p>Seamless safety review by IDMC when 8 patients have undergone two fimaCHEM treatments</p>	<ul style="list-style-type: none"> <li>• IDMC = Independent Data Monitoring Committee</li> </ul>
<p>Event driven interim analysis after 60 progression free survival (PFS) events</p>	<ul style="list-style-type: none"> <li>• Interim analysis expected approximately 36 months after inclusion of first patient in May 2019</li> </ul>
<p>Timing and format for study conclusion may be impacted by outcome of Interim analysis</p>	<ul style="list-style-type: none"> <li>• Final analysis expected approximately 50 months after inclusion of first patient in May 2019</li> </ul>

# BILE DUCT CANCER – CLINICAL PHASE I STUDY

- ▶ Dominated by significant target tumour reduction in the first 6 months
- ▶ Best Overall Response – all patients in all cohorts with measurable disease follow-up (n=15)

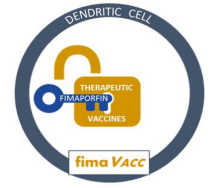


# BILE DUCT CANCER – PHASE I DOSE-ESCALATION STUDY

► Positive early signs of efficacy – median Overall Survival of 21.7 months at selected dose

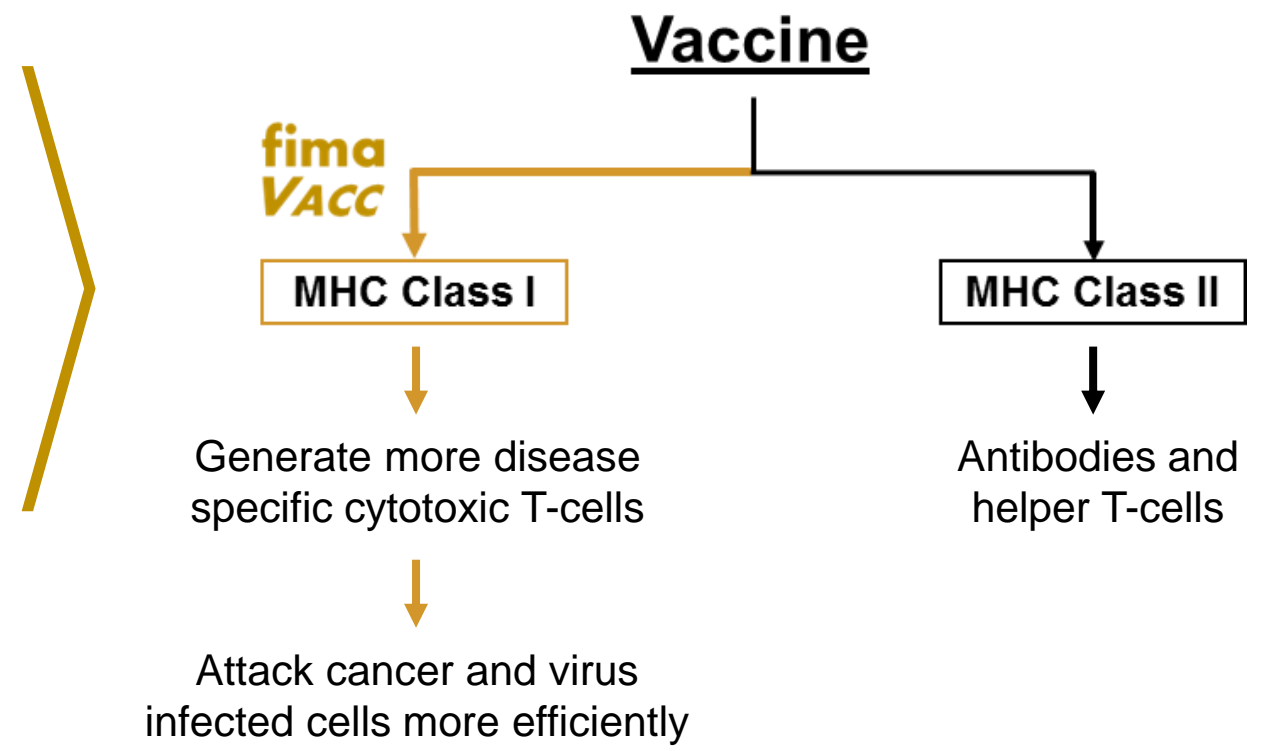
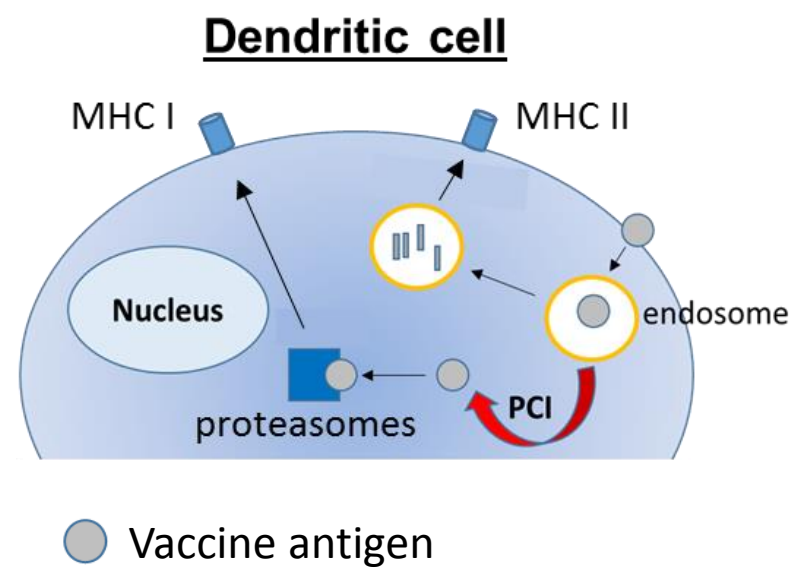
Parameters	Cohort IV (N=6)	Phase I – all dose-escalation cohorts (N=16)
Objective Response Rate (ORR)	3/5 patients (2 PR; 1 CR)	4/12 patients (2 PR; 2 CR)
Median Overall Survival (mOS)	21.7 months	14.4 months

- Cohort IV dose has been selected for the pivotal RELEASE study
- Half of the patients in Cohort IV survived >30 months
- One patient in Cohort IV still alive by mid-November, 44 months after treatment
- Encouraging Phase I results paved the way for a pivotal trial, with interim analysis
- Safety of two treatments provided in a Phase I Extension



# PCI TECHNOLOGY

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



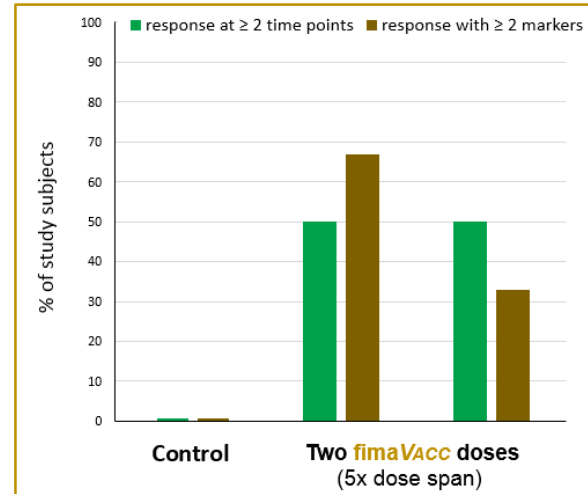
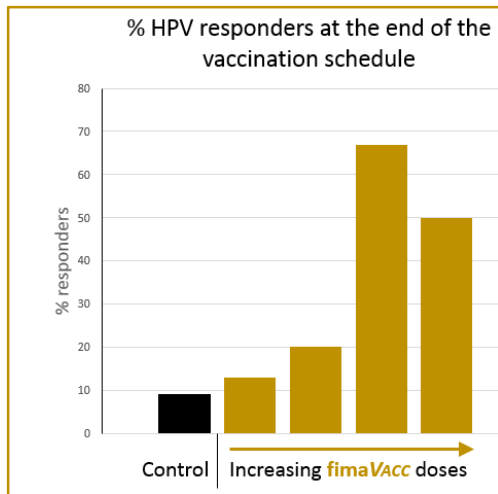


## SOLID PROGRESS OF THE **fima VACC** PROGRAMME IN 2019

- ▶ Successful clinical proof-of-concept
  
- ▶ Phase I study provided successful clinical proof-of-concept for **fima VACC**
  - ▶ Overall objective to determine the safety, tolerability and immune response of **fima VACC**
  - ▶ Proof of concept and efficacy in terms of intradermal dosing in humans
  - ▶ Positive overall characterisation of tolerability, with efficacy seen across a wide tolerable dose span

# SUCCESSFUL CLINICAL PROOF-OF-CONCEPT

▶ Phase I study in healthy volunteers shows enhanced immune responses



**fima VACC** provides:

- ✓ *Increased number of responders*
- ✓ *Enhanced T-cell responses*
- ✓ *Improved T-cell functionality*

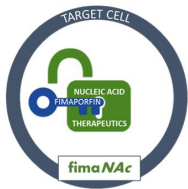
- ▶ Results show that **fima VACC** induces:
  - ▶ Substantial increase in number of T-cell responders to HPV E7 peptides
  - ▶ Clearly enhanced overall T-cell responses
  - ▶ More robust CD8 T-cell responses, which are notoriously difficult to induce with E7
  - ▶ Increased functionality of the induced CD8 T-cells
  
- ▶ **Highly sought-after features – especially for therapeutic vaccination**

## SOLID PROGRESS OF THE fima VACC PROGRAMME

- ▶ Next steps
  - ▶ Study results presentation – ESMO Immuno-Oncology Congress in Dec 2019
  - ▶ Assessing publication in scientific journal
  - ▶ Phase I results to be used in direct partnering efforts and plan for clinical proof-of-concept in disease setting



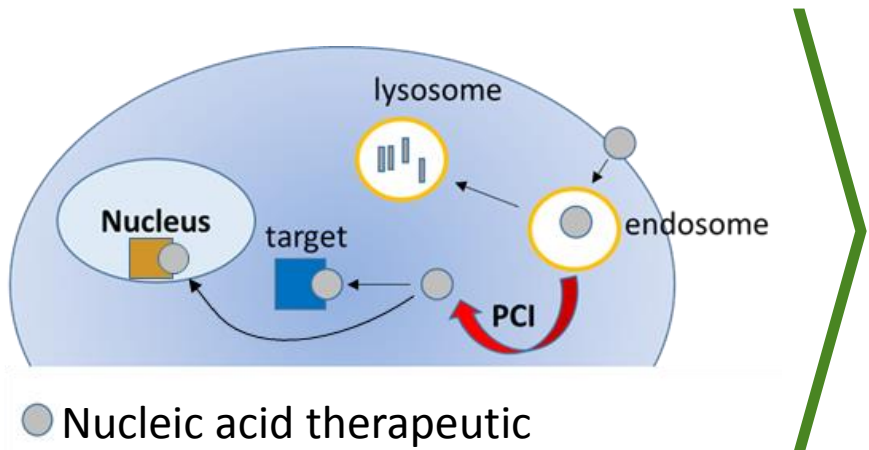
*Patented disposable "band-aid-like" device for user-friendly illumination of the vaccination site*



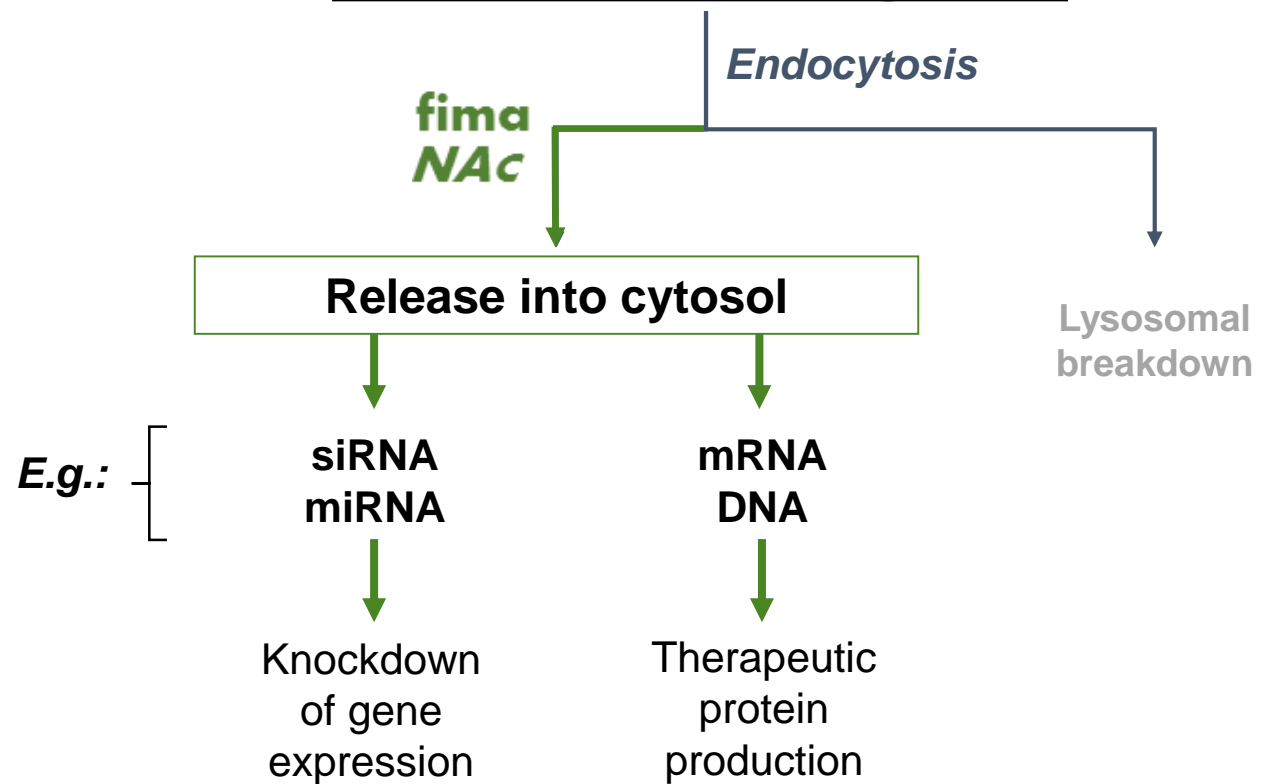
# PCI TECHNOLOGY

► **fimaNAC** – mode of action

## Target cell



## Nucleic acid therapeutic



## RESEARCH COLLABORATIONS

▶ Six collaborations established with key players in nucleic acid therapeutics

- ▶ Astra Zeneca collaboration extended to end of 2019 – scope expanded to evaluate whether synergies established in oncology are transferrable to other disease areas – additional 6 months period for determination of potential next steps
- ▶ Promising response on patent application for mRNA delivery, which is highly relevant for several collaboration partners

fimaNAC

AstraZeneca 

IMV™  
IMMUNOVACCINE

  
BAVARIAN NORDIC

  
eTheRNA

  
Phio  
PHARMACEUTICALS

BIONTECH 

# FINANCE

- ▶ Key financial figures
- ▶ Other income in line with previous year
- ▶ Operating result impacted by planned start-up activities and initiation of the RELEASE study

(figures in NOK 1,000)	Q3 2019	Q3 2018	YTD 2019	YTD 2018	FY 2018
Other income (public grants)	2 425	2 238	7 275	6 613	9,585
Operating results	-18 495	-8 386	-63 474	-30 241	-44 519

(figures in NOK 1,000)	Q3 2019	Q3 2018	YTD 2019	YTD 2018	FY 2018
Net change cash and cash equivalents*	-17 288	-7 869	-64 993	-30 253	298 537
Cash and cash equivalents	284 332	20 536	284 332	20 536	349 326

\*Including effects from exchange rate fluctuation on bank deposits in EURO from October 2018

# KEY ACHIEVEMENTS & NEAR-TERM MILESTONES

1H 2019	✓ <b>fimaVACC</b>	Completion of Phase I immune analyses
1H 2019	✓ <b>fimaCHEM</b>	Safety of repeated treatment confirmed
1H 2019	✓ <b>fimaCHEM</b>	First patient enrolled in the RELEASE study
2H 2019	✓ <b>fimaVACC</b>	Phase I results presented at key conference
1H 2020	✓ <b>fimaVACC</b>	Phase I results published in scientific journal
1H 2020	✓ <b>fimaCHEM</b>	First US patient enrolled in the RELEASE study
2H 2020	✓ <b>fimaCHEM</b>	First Asian patient enrolled in the RELEASE study

# INVESTMENT HIGHLIGHTS

## Broad platform technology

PCI is a 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 candidate

## Advanced lead product candidate

**fima CHEM** – Amphinex® is an orphan designated (EU & US) first-in-class product candidate in pivotal development for treatment of bile duct cancer – a disease without approved drugs

## Encouraging 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

## Defined development 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

## Pipeline opportunities

**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

## Experienced leadership

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



## FOR ENQUIRIES

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