



# PCI BIOTECH

Unlocking the potential of innovative medicines

**BioEquity Europe 2018**

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# PCI BIOTECH AT A GLANCE

## ► Unlocking the potential of innovative medicines

Biopharmaceutical company focusing on development and commercialization of novel therapies for the treatment of cancer

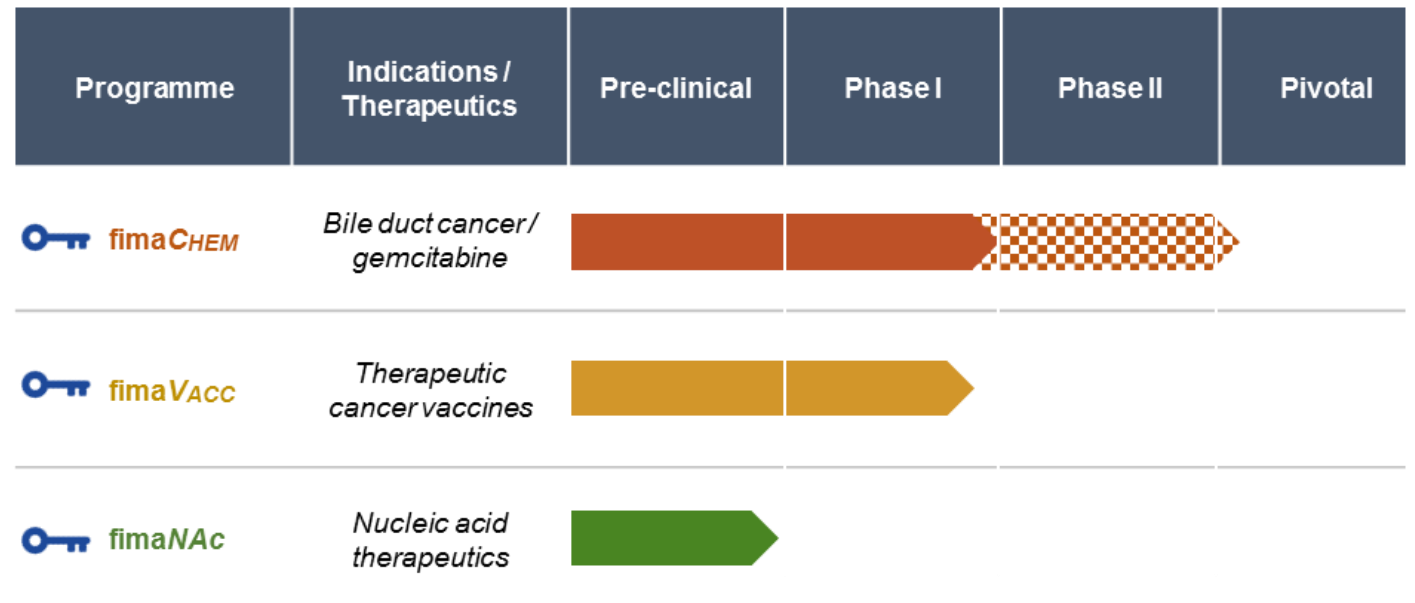
Leverages Photochemical Internalisation ('PCI') technology, originating from the Oslo University Hospital – the Radium Hospital

Platform technology with three programmes targeting an attractive and growing oncology market

Lead programme, **fimaCHEM**, 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

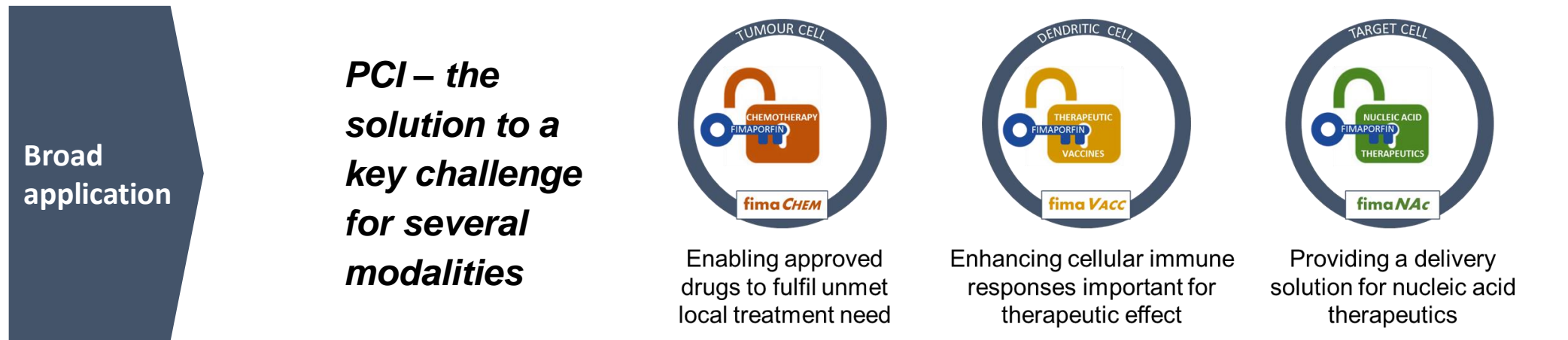
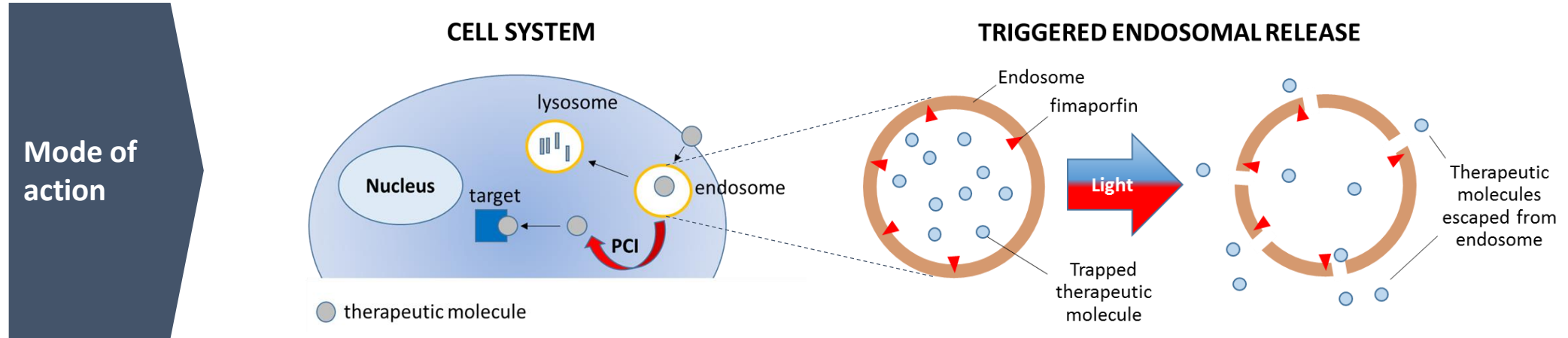
Listed on Oslo Børs (PCIB), M-Cap Euro ~130m

### *An oncology focused company with three well differentiated assets*



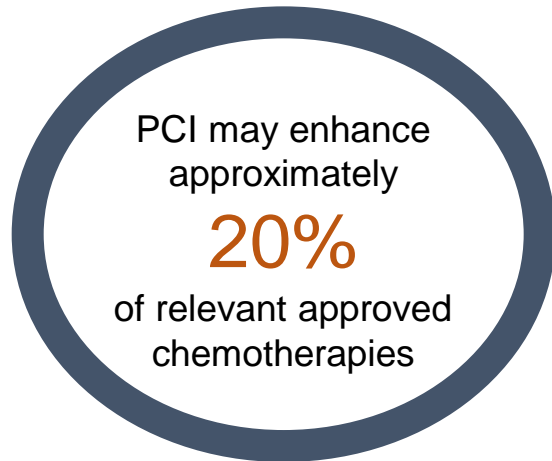
# PCI TECHNOLOGY

► Enabling drugs to reach intracellular therapeutic targets



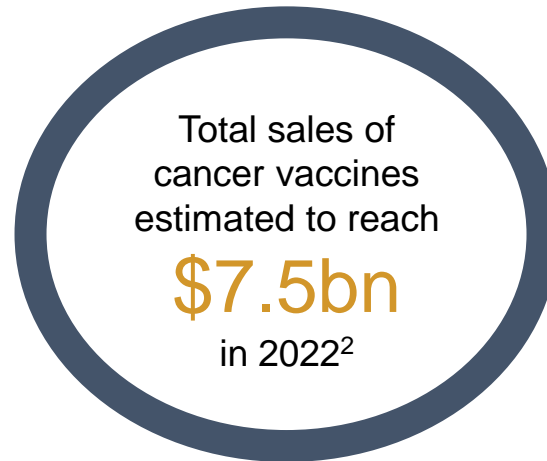
# THREE WELL-DEFINED DEVELOPMENT PROGRAMMES

## 1 fimaCHEM



- ▶ First-in-man study published in Lancet Oncology<sup>1</sup>
- ▶ Promising tumour responses in Phase I in inoperable extrahepatic bile duct cancer
- ▶ Pivotal phase ready, with potential for approval based on interim read
- ▶ Orphan disease with high price potential

## 2 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

## 3 fimaNAC

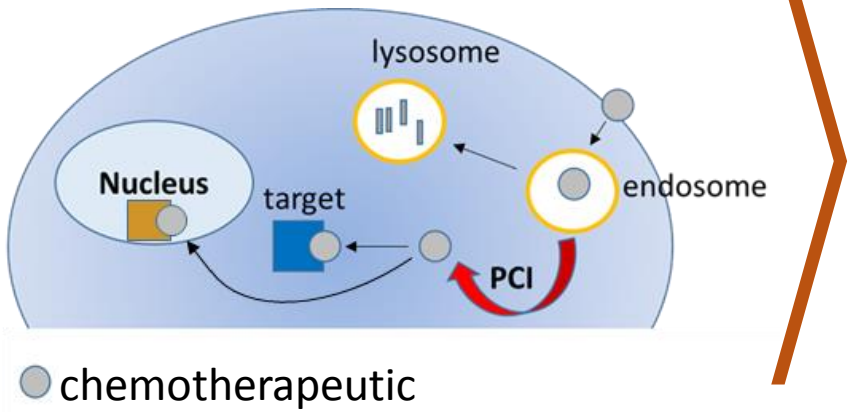


- ▶ Estimated sales of \$18bn in 2030<sup>3</sup> (RNAi alone)
- ▶ Opportunistic collaborative approach
- ▶ Aim is to out-license the technology on non-/semi-exclusive basis

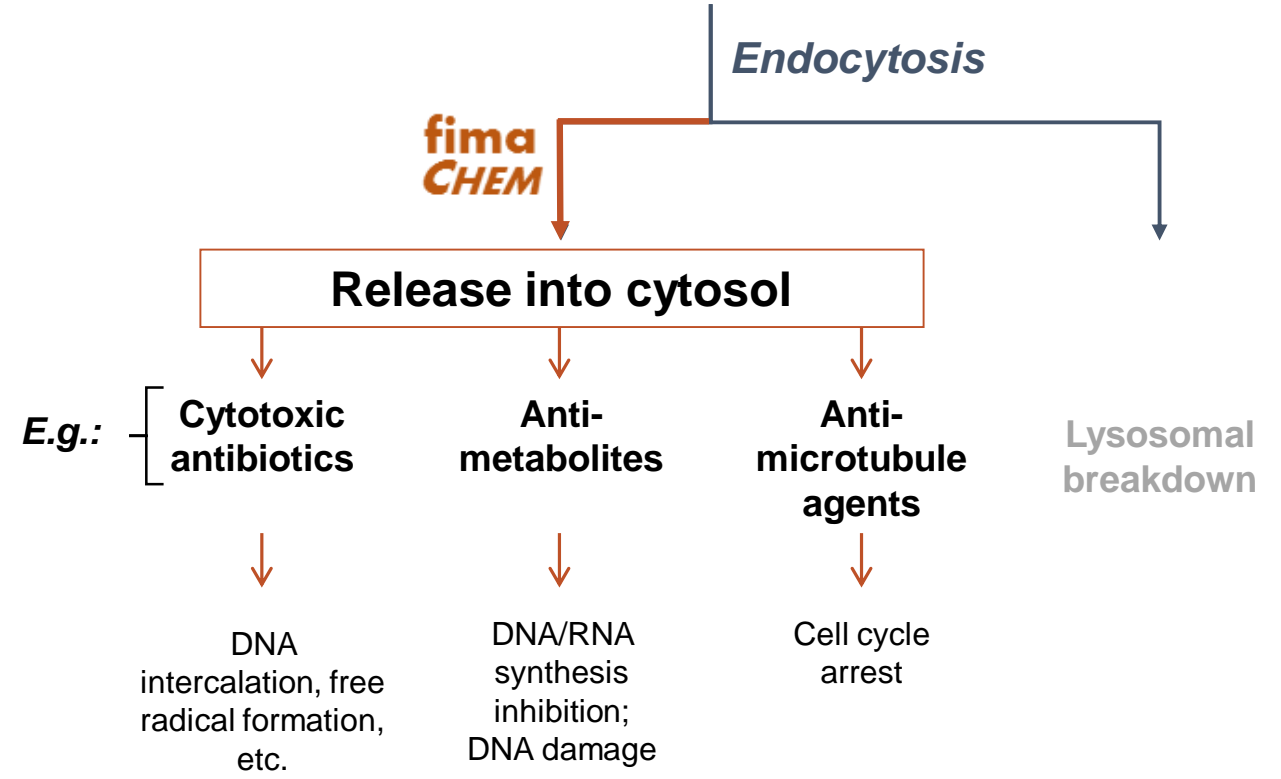
# PCI TECHNOLOGY

► **fimaCHEM** – mode of action

## Cancer cell



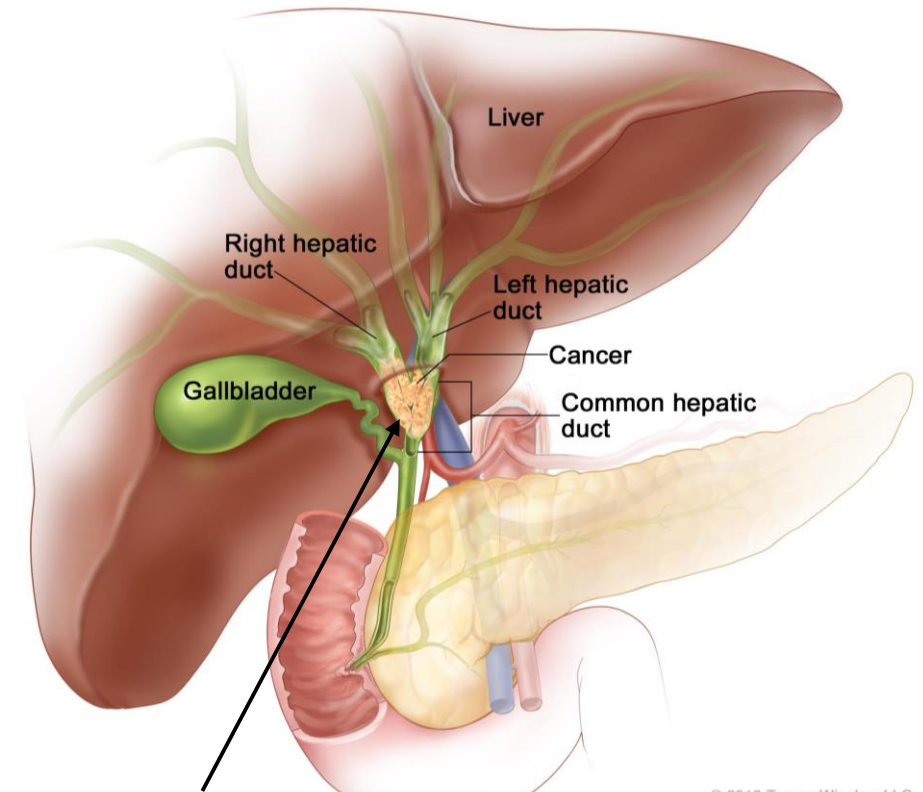
## Chemotherapeutics



# BILE DUCT CANCER

## ► Location and classification

- Often referred to as cholangiocarcinoma
- The cancer cells originates from the cells inside the bile duct (called cholangiocytes)
- Cholangiocarcinoma includes:
  - Intrahepatic tumours (10%<sup>1</sup>)
  - Perihilar tumours (60-70%<sup>1</sup>)
  - Distal tumours (20-30%<sup>1</sup>)
  - Different incidence, pathobiology and management



*Perihilar bile duct cancer is the initial target for PCI treatment*

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# 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<sup>1</sup> – higher in Asia
- ▶ Five-year survival rate of less than 5% and almost 0% when inoperable<sup>1</sup>
- ▶ Average survival inoperable: ≈12 months<sup>2</sup>
- ▶ Current management<sup>1</sup>
  - 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

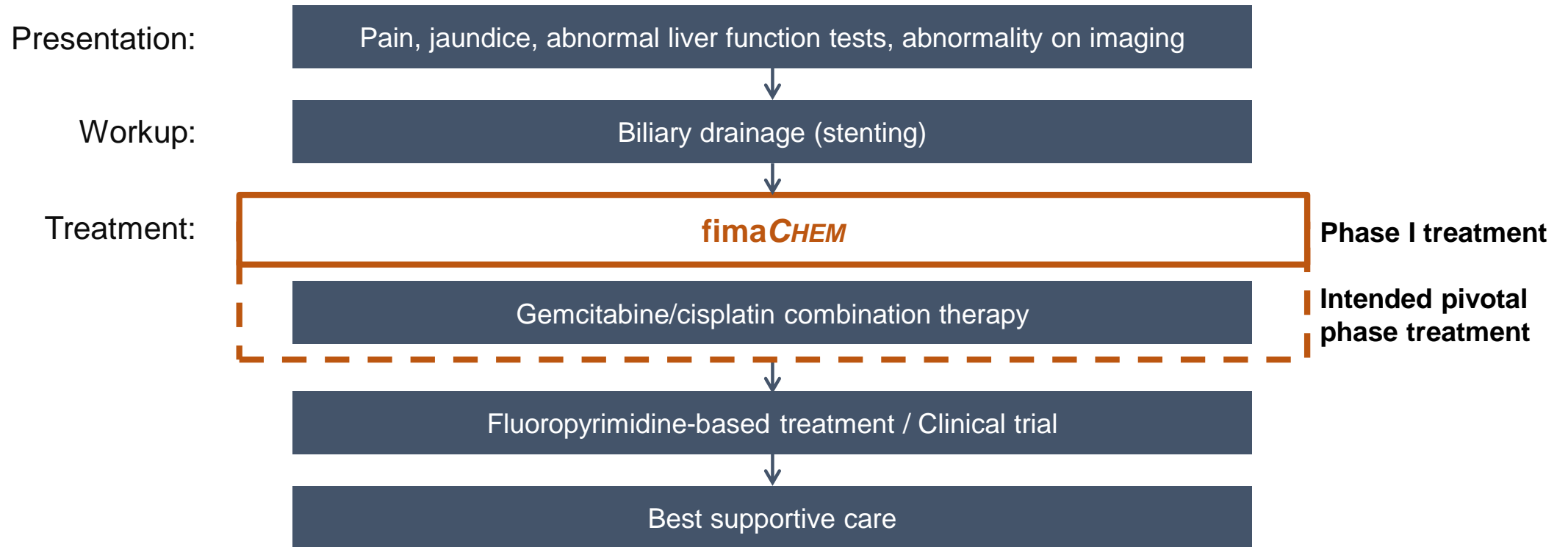
- Preclinical and clinical data supports the notion of potential abscopal effects with **fimaCHEM**
- May be ideal for combination with checkpoint inhibitors



# INOPERABLE EXTRAHEPATIC BILE DUCT CANCER

## ► An underserved patient population

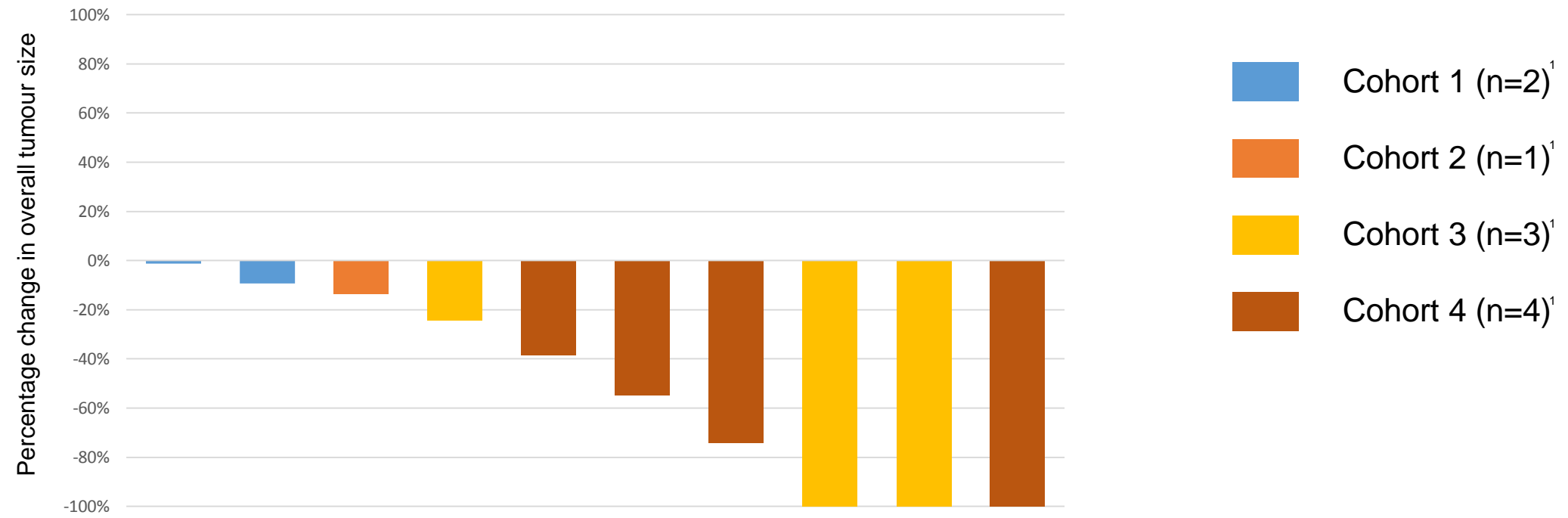
- No approved medical treatment
- Combination therapy with gemcitabine and cisplatin recommended



# BILE DUCT CANCER – CLINICAL PHASE I/II STUDY

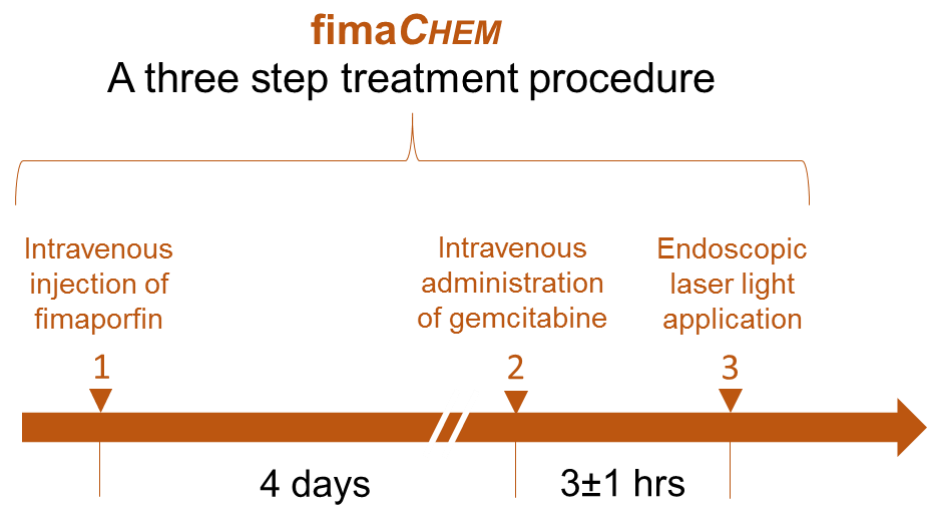
## ► Encouraging early signs of efficacy in Phase I

- ★ 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
- ★ Best overall response (all radiologically evaluable patients) – almost all showed tumour reduction

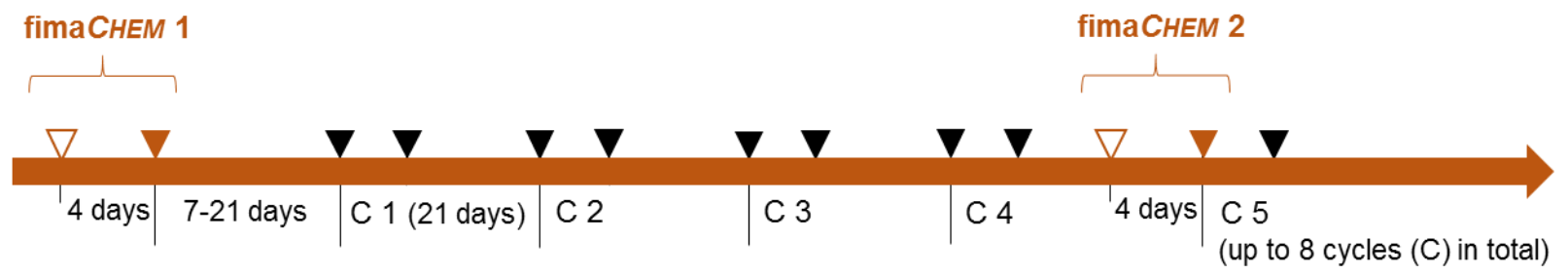


# BILE DUCT CANCER – PHASE I EXTENSION STUDY

▶ 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 preparation for the next phase



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

# BILE DUCT CANCER

▶ A sizeable orphan market potential

▶ **Immediate target market is as first line treatment**

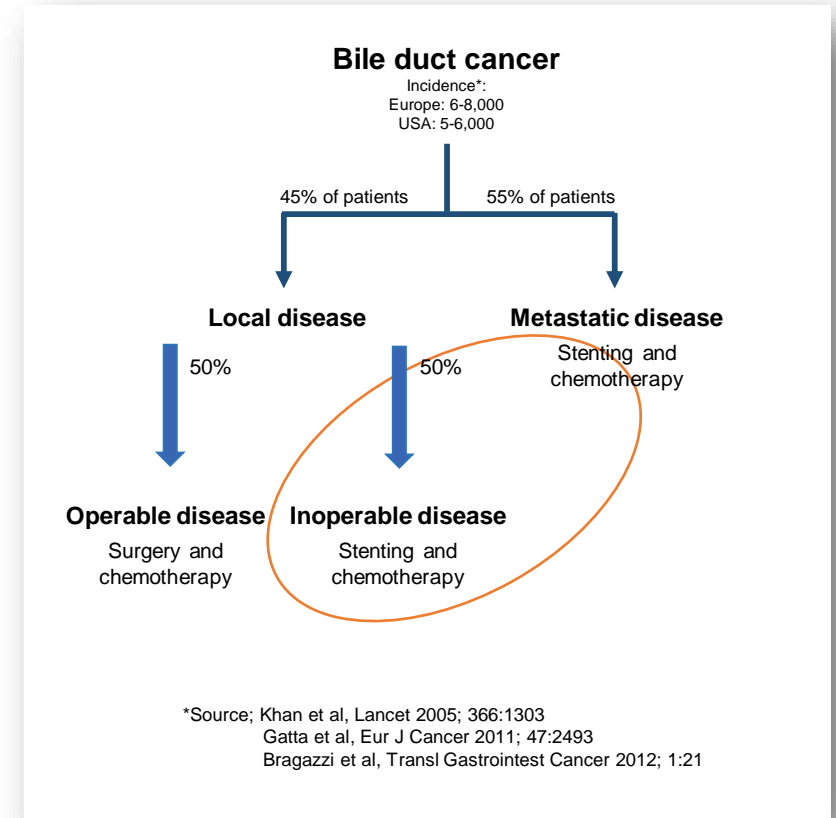
- Incidence is close to 15,000 across Europe and the US
- Immediate target is inoperable patients with local disease
- Approximately 3,000 assumed to be eligible for **fimaCHEM**
- Possible upside in distal and more advanced metastatic disease
- Higher incidences in Asia

▶ **Attractive price potential**

- Lack of approved medicinal treatment options
- Diseases with <10,000 in US support annual pricing >\$100,000<sup>1</sup>

▶ **Potential significant majority share of the market**

- Anticipated benefits
  - No competing marketable treatment alternatives
  - Greater efficacy due to local chemotherapy boost
  - Easy light access through established standard procedures



# 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 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

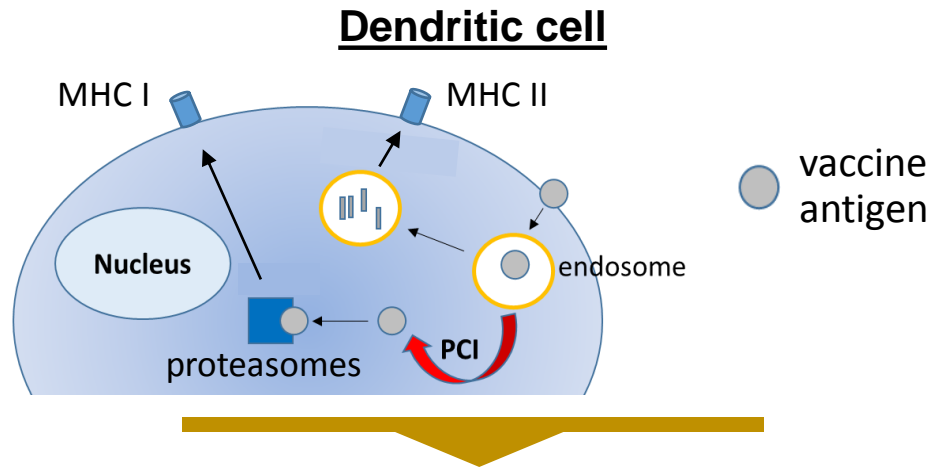
- Single randomised pivotal study with potential for accelerated / conditional approval based on interim analysis

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

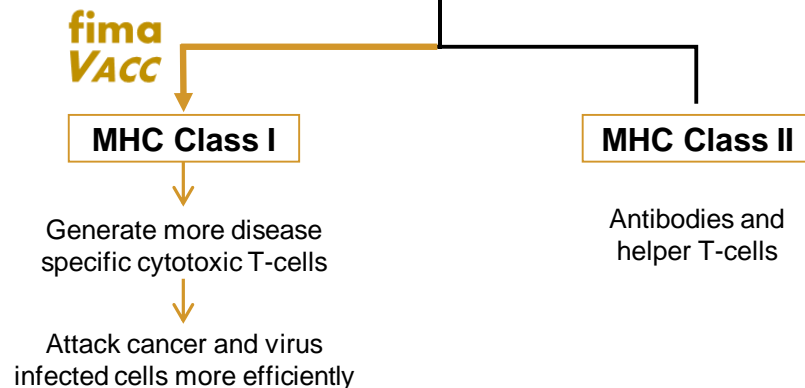
- Full study design to be announced upon completion of clinical advisory interactions

# PCI TECHNOLOGY

## ► **fima VACC** – mode of action



## Vaccine



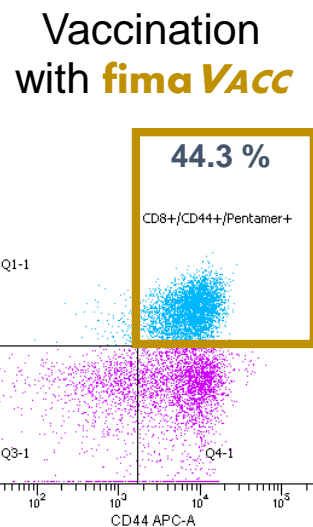
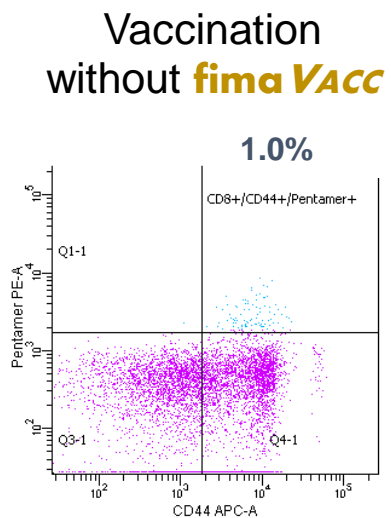
## ► **fima VACC** – strong potential

- Opportunity to play a key role in second generation immunotherapy
- 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
  - Prophylactic & therapeutic vaccination
- Excellent stability
  - Few logistical challenges (stable at room temperature in solution and can be autoclaved)
- Important recent IP generation

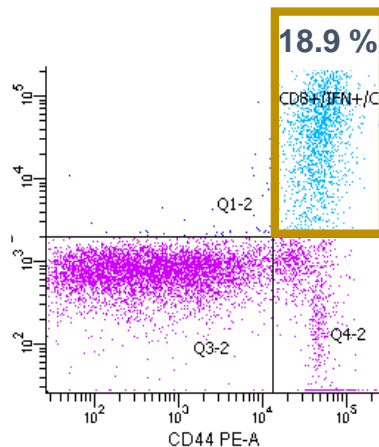
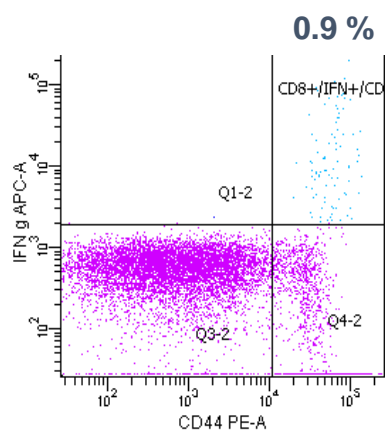
# fima VACC STRONGLY ENHANCES VACCINATION EFFECTS

► Impressive effects with clinically relevant HPV therapeutic vaccine in mice

Amount of activated antigen-specific CD8 T-cells in blood



Amount of activated antigen-specific CD8 T-cells in spleen



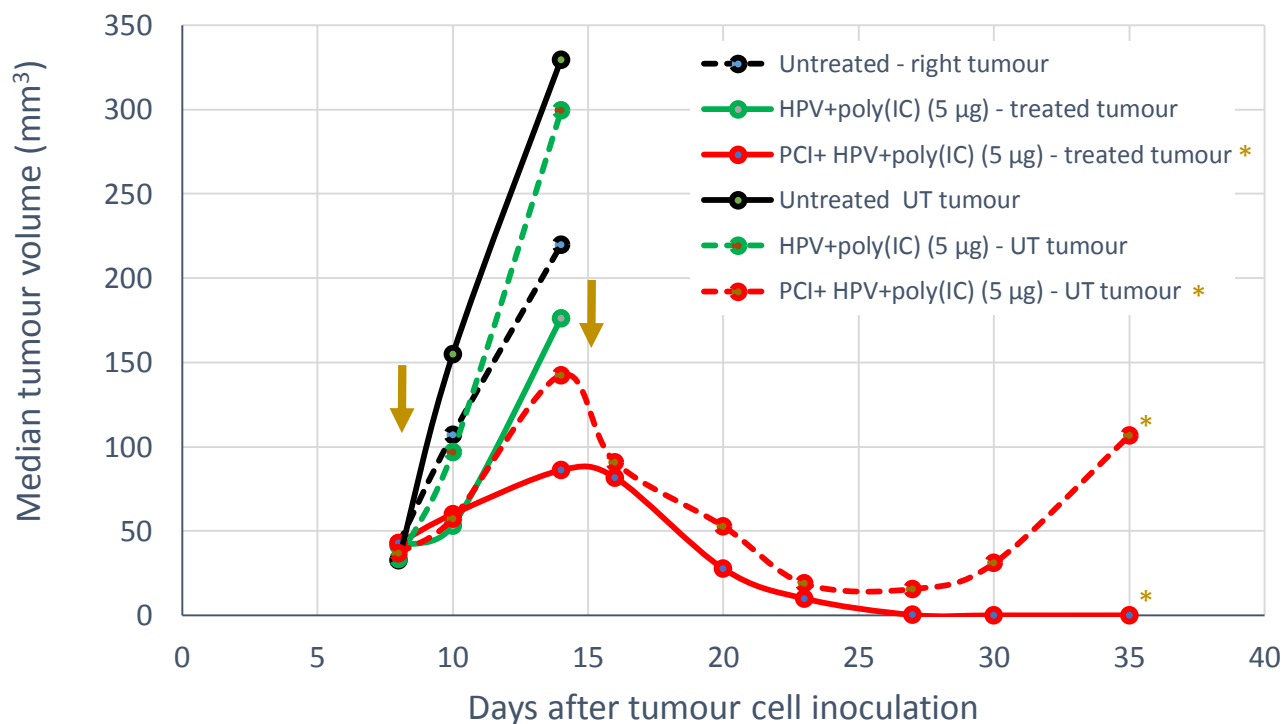
## Cytotoxic (CD8) T-cells

- Most important immune cells to fight tumours
- Difficult to induce with vaccination
- **fima VACC** strongly enhances the ability of vaccines to induce CD8 T-cells:
  - >20 and >40 times enhancement seen in spleen and blood cells, respectively
  - Generation of immunological memory

# INTRA-TUMOURAL VACCINATION WITH **fima VACC**

▶ Eradicates treated tumour and induces strong response in non-treated tumour

Two tumours in TC-1 model. Median tumour volume of at least 4/6 animals in each group



▶ Two tumours inoculated simultaneously

▶ Intra-tumoural immunisation (↓) generates an immune response capable of destroying untreated tumours

\* **fima VACC** treated animals  
 UT: untreated



# 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 treated
  - 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 examples available – near-term focus on characterisation of the immune response
  - Part 3 TBD
  - Expected study completion: 2H 2018

Vaccination features:	<b>fima VACC</b>
<i>Enhanced T-cell blood levels</i>	<input checked="" type="checkbox"/>
<i>High T-cell response rates</i>	<input checked="" type="checkbox"/>
<i>Early T-cell responses</i>	<input checked="" type="checkbox"/>

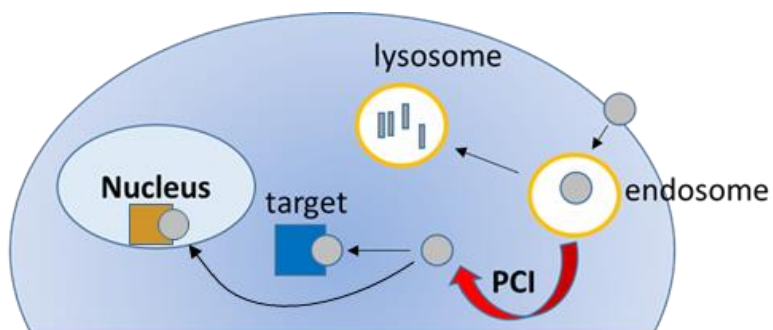


*Patented disposable “band-aid-like” device for user-friendly illumination of the vaccination site*

# PCI TECHNOLOGY

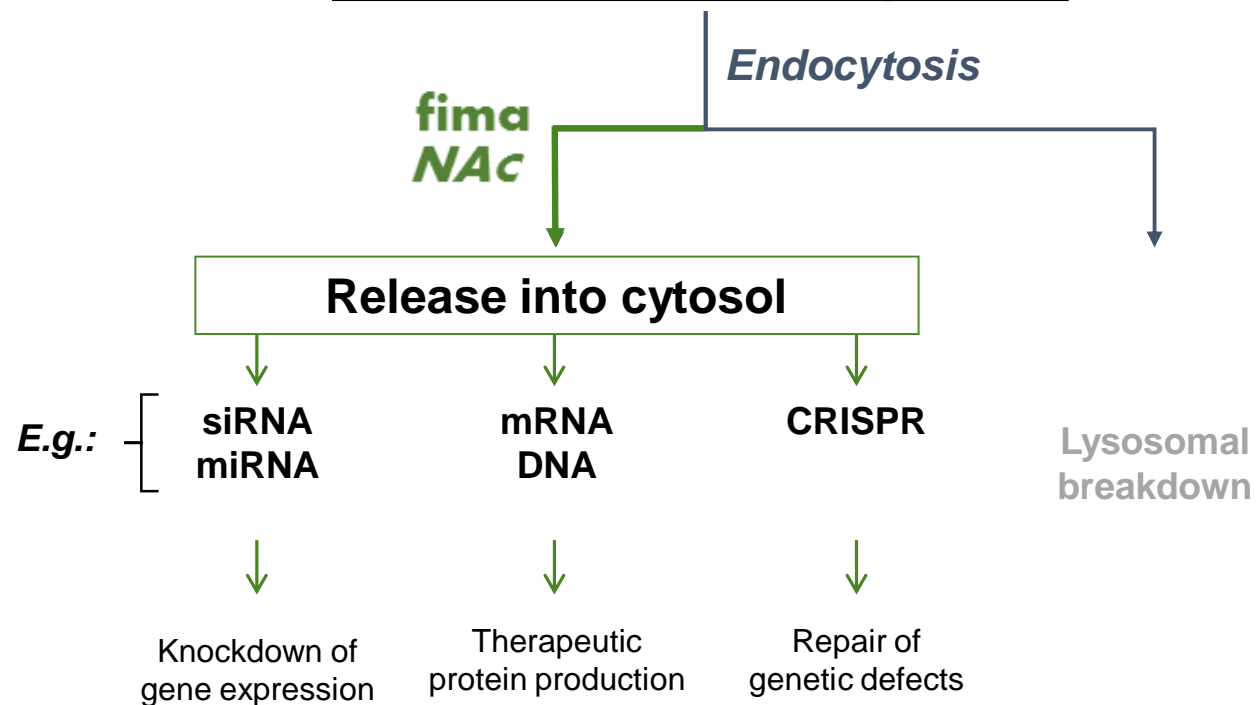
► **fimaNAC** – mode of action

## Cancer cell



● Nucleic acid therapeutic

## Nucleic acid therapeutic



# RESEARCH COLLABORATIONS

► Five active collaborations within vaccination and nucleic acid therapeutics

fima VACC



- Collaboration initiated 1Q 2016
- Norwegian immunotherapy company
- Therapeutic cancer vaccine against human telomerase



- Collaboration initiated 2Q 2015
- Listed on Nasdaq, developing innovative therapeutic siRNA
- Collaboration expanded to immuno-oncology following RXi's MirlImmune acquisition

Top-10  
large  
pharma

- Collaboration initiated 3Q 2015
- A global leader in nucleic acid therapeutics
- Collaboration expanded to include *in vivo* studies and duration to end 2Q'18

fima NAc



- Collaboration initiated 3Q 2016
- German biotech company developing individualised cancer immunotherapies
- Clinical programmes in melanoma, head & neck, breast, ovarian and pancreatic cancer



- Collaboration initiated 4Q 2016
- A global leader in mRNA-based immunotherapies
- Evaluate synergistic effects between companies' technologies

Aim to evaluate synergies between the fima platform and partner technologies, with the potential for further partnerships

# GOOD PROGRESS AND EXCITING OUTLOOKS

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fimaCHEM

## Progressing development in bile duct cancer

- Encouraging tumour response and emerging survival data from Phase I
  - Fastest way to market determined through regulatory interactions with authorities
  - Preparations for pivotal phase progressing towards initiation in second half of 2018
- 

fimaVACC

## Clinical validation of the vaccination technology

- Initial results suggest overall T-cell enhancement at tolerable doses, as well as early responses and high response rates
- 

fimaNAC

## Progressing the research collaborations

- Top-10 pharma collaboration extended to end of first half 2018 and entered into *in vivo* studies

# PCI BIOTECH HOLDING ASA

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