



PCI BIOTECH

Unlocking the potential of innovative medicines

Q2 & 1H 2019 PRESENTATION

August 28, 2019

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PCI BIOTECH

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HIGHLIGHTS

► First half-year 2019

fima *CHEM*

- First patient enrolled in the RELEASE study
- Regulatory and ethics approvals for the RELEASE study achieved in two thirds of the planned countries, including USA
- Almost half of the RELEASE study sites opened and actively screening for patients
- Initiated feasibility study in Asia with the aim of including sites in 2020
- Completion of the full Phase I study, with successful safety read-out for repeated treatment
- Presented Phase I data at key conferences in Asia-Pacific and US

HIGHLIGHTS

► First half-year 2019

fima VACC

- Successful clinical proof-of-concept with enhanced immune responses
- Preclinical publication in high-impact immunology journal (subsequent event)

fima NAc

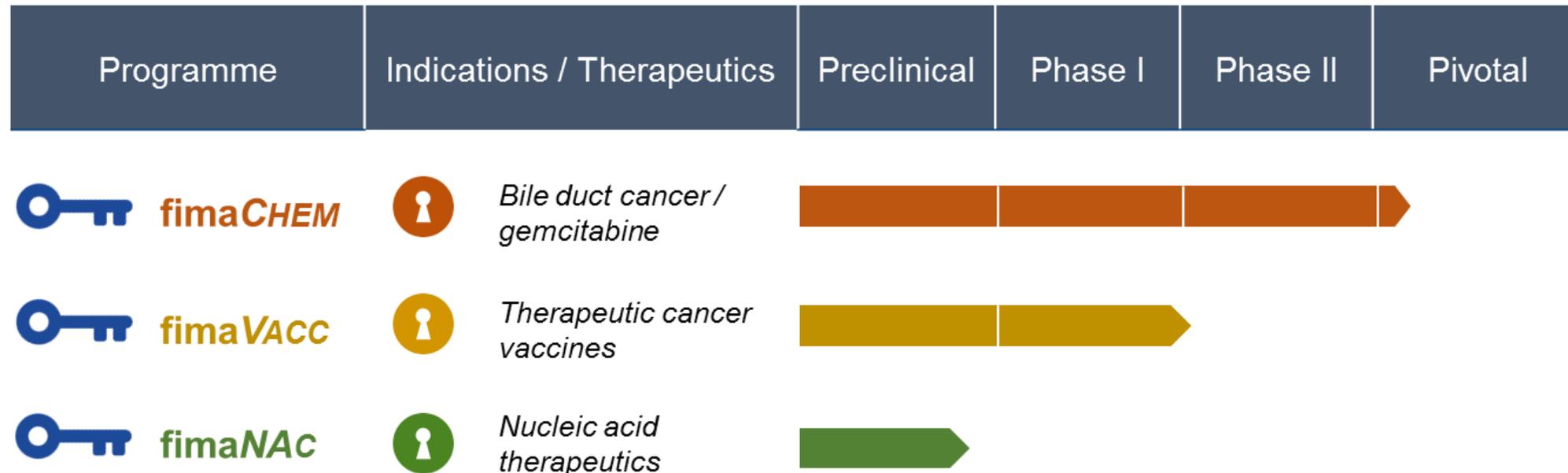
- Promising response on patent application for mRNA delivery (subsequent event)
- Final extension of the top-10 pharma research collaboration (subsequent event)

Corporate

- Further strengthened the Scientific Advisory Committee and the Board of Directors

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

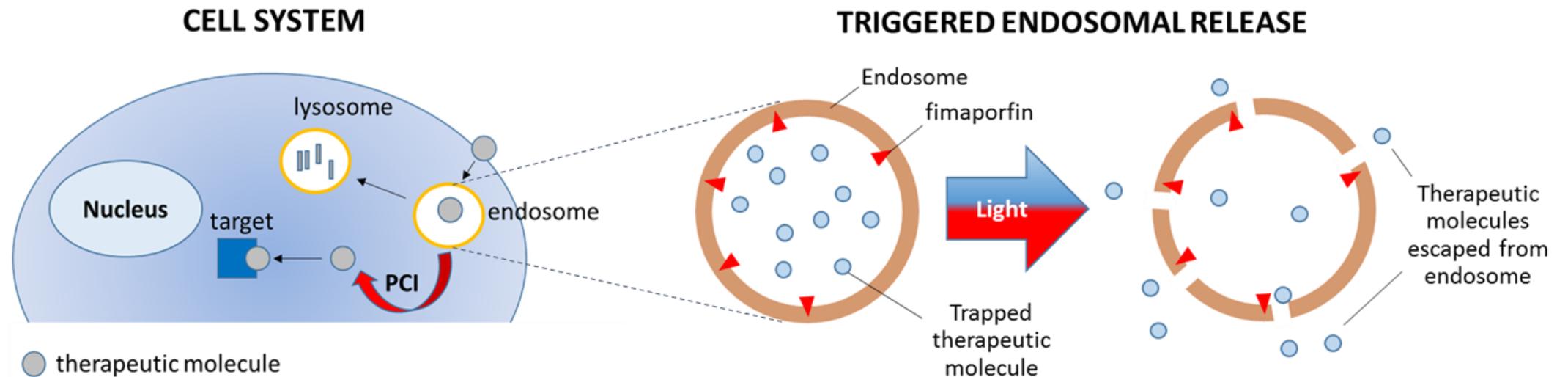


An oncology focused company with three well differentiated assets

PCI TECHNOLOGY

- ▶ Enabling drugs to reach intracellular therapeutic targets

Mode of action



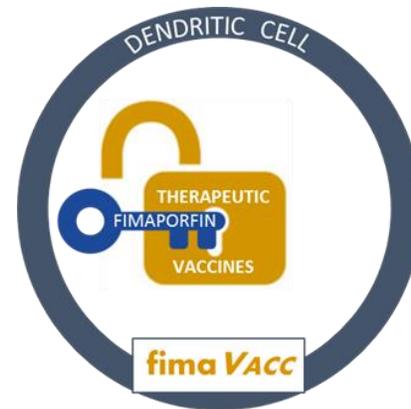
PCI TECHNOLOGY

- ▶ Enabling drugs to reach intracellular therapeutic targets

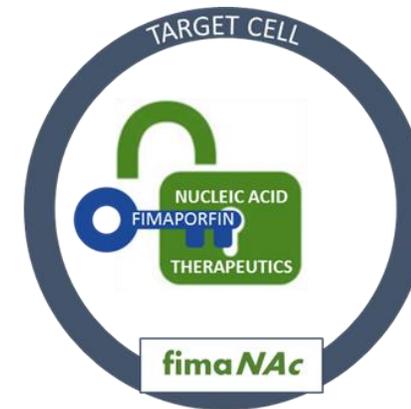
PCI – the solution to a key challenge for several modalities



Enabling approved drugs to fulfil unmet local treatment need



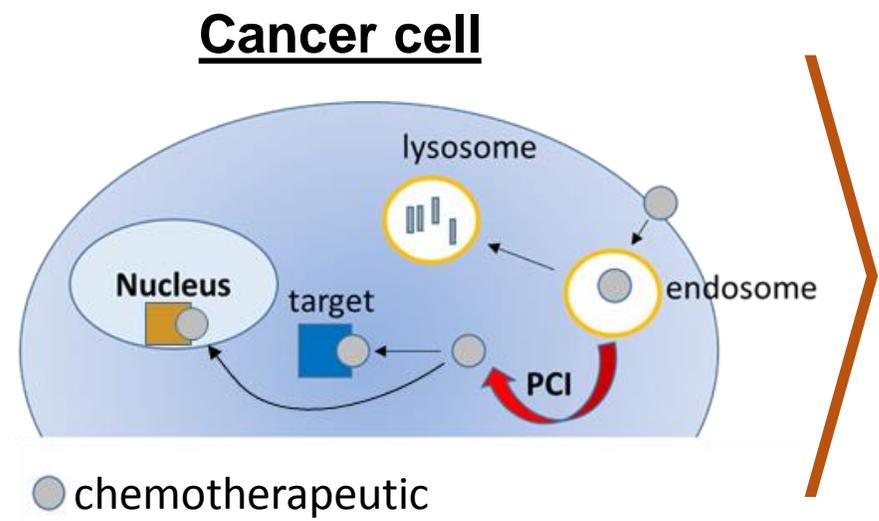
Enhancing cellular immune responses important for therapeutic effect



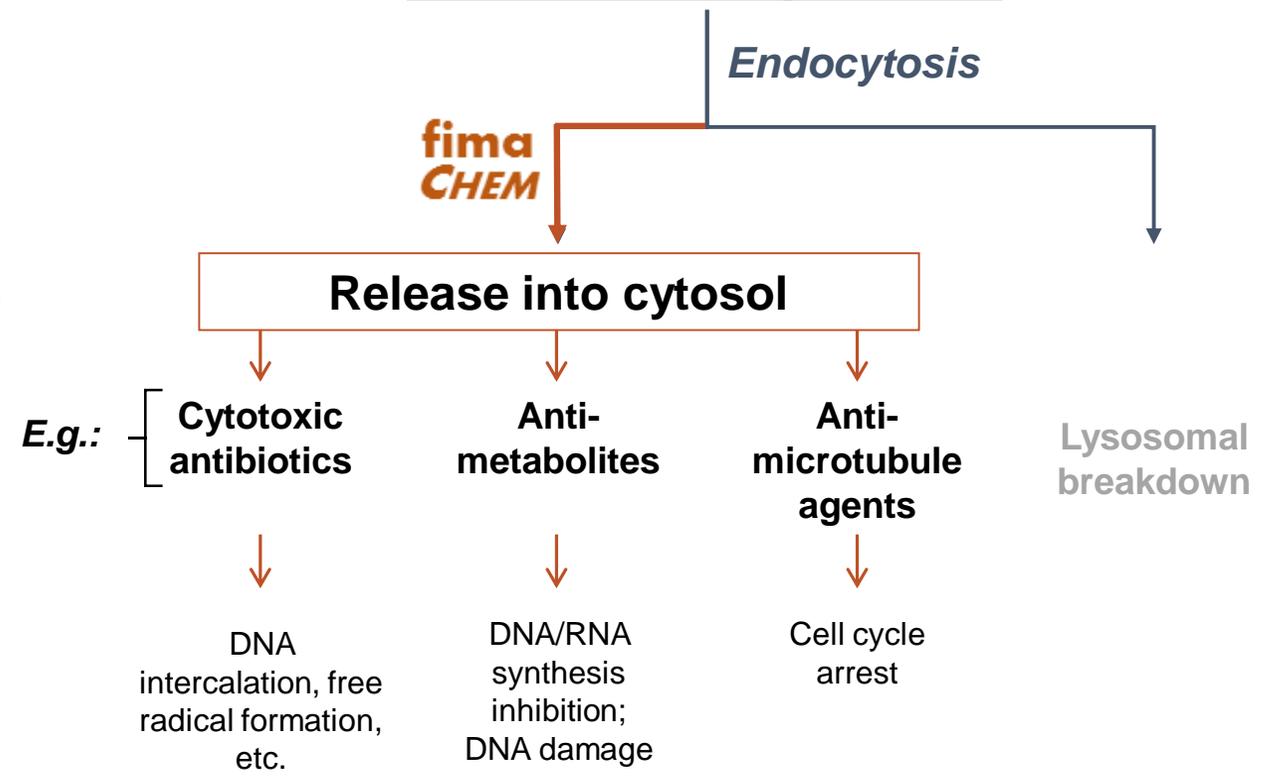
Providing a delivery solution for nucleic acid therapeutics

PCI TECHNOLOGY

► **fimaCHEM** – mode of action



Chemotherapeutics



BILE DUCT CANCER – EXTRAHEPATIC INOPERABLE

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

- ▶ Orphan indication
- ▶ Average survival inoperable: 11-12 months¹
- ▶ 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

¹ N Engl J Med 2010;362:1273-81

BILE DUCT CANCER – PHASE I DOSE-ESCALATION STUDY

► Cohort IV is selected dose for pivotal study – limited but encouraging data

Positive early signs of efficacy – mOS of 21.7 months at selected dose in Cohort IV

Parameters	Cohort IV (N=6) (0.25mg/kg)	Phase I – all dose-escalation cohorts (N=16) (0.06-0.25mg/kg)
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

- Half of the patients in Cohort IV survived >30 months
- One patient in Cohort IV alive by end June, more than three years after treatment

BILE DUCT CANCER – PHASE I Extension STUDY

▶ Extension cohort confirmed safety of repeated treatment

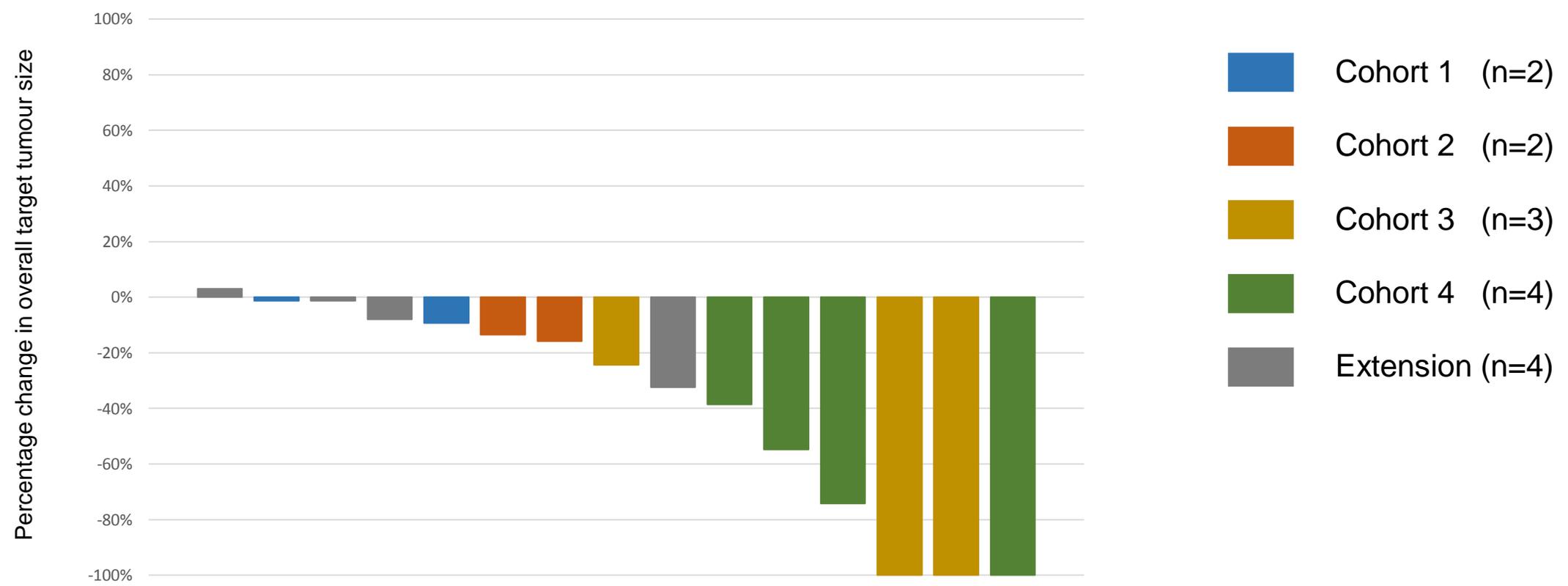
▶ **Safety endpoint reached – pivotal study is performed with up to two treatments**

- A total of seven patients were included – five received two treatments
- Four patients had radiologically measurable disease
- The average tumour burden in the extension was about twice that in the dose escalation
- The interim median overall survival (mOS) for all the patients receiving the pivotal study dose (N=13) is by end June approx. 15 months, with two patients still alive (outcome range up to 15.6 months)

BILE DUCT CANCER – CLINICAL PHASE I STUDY

▶ Dominated by significant target tumour reduction in the first 6 months

▶ **Best Overall Response – all patients with measurable disease in all cohorts including extension (n=15)**



BILE DUCT CANCER – RELEASE STUDY

▶ Pivotal study progressing well

- ▶ **First patient included in May – recruitment progressing according to plan**
- ▶ **Achieved safety endpoint in the extension study – RELEASE initiated with up to two treatments**
- ▶ **Regulatory and ethics approvals progressing according to plan – by mid-August approvals had been received for USA and 8 of 11 planned European countries: Norway, Germany, France, Spain, Belgium, Poland, Sweden and Denmark**
- ▶ **Site initiations progressing according to plan with a total of 15 sites across 7 European countries open for enrolment and actively screening for patients by mid-August**
- ▶ **Presentation of Phase I data at the US CCA Foundation annual conference in USA (Jan'19), at the 3rd Asia-Pacific CCA conference in Taiwan (Mar'19) and at the 17th International Photodynamic Association World Congress in USA (Jun'19)**

BILE DUCT CANCER – RELEASE STUDY

- ▶ Randomised study with interim analysis for potential accelerated/conditional approval

- ▶ **Orphan designation granted in both the US and EU**
- ▶ **Fastest way to market determined through regulatory interactions with authorities**

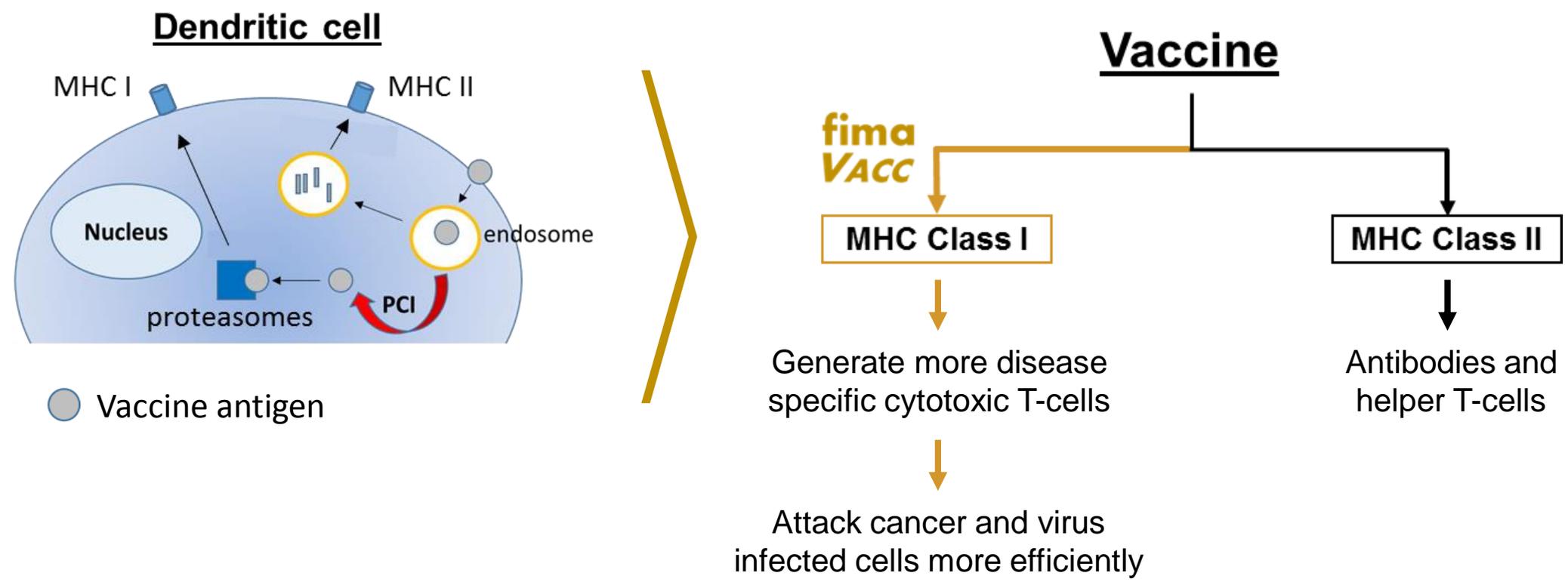
- First line treatment of patients with inoperable extrahepatic bile duct cancer
- Approx. 40 key hospitals (Europe & USA)
- Approx. 36 months to interim and 50 to final analysis
- Randomisation (1:1) of 186 patients
- Primary endpoint: PFS^a, with OS^b as key secondary
- Interim analysis primary endpoints: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

- ▶ **The prevalence of bile duct cancer is higher in Asia than in the western world**
- ▶ **Feasibility study in Asia ongoing to select the most appropriate RELEASE study sites for patient recruitment and market impact, with the aim to open sites in 2020**

^aPFS: Progression Free Survival; ^bOS: Overall Survival; ^cORR: Objective Response Rate; ^dIDMC: Independent Data Monitoring Committee

PCI TECHNOLOGY

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



FURTHER MECHANISTIC UNDERSTANDING OF **fima VACC**

▶ Preclinical publication in high-impact immunology journal

- ▶ A preclinical study performed in collaboration with University Hospital Zürich has been published in the high-impact immunology journal “Frontiers in Immunology”:
 - *Combined photosensitisation and vaccination enable CD8 T-cell immunity and tumor suppression independent of CD4 T-cell help. Varypataki et al., Front. Immunol. 10:1548*
- ▶ The study provided important data further contributing to the understanding of the mechanism behind **fima VACC** induced immune responses.
- ▶ Strong CD8 T-cell activation and tumour regression was seen after vaccination with **fima VACC** in melanoma bearing mice, including mice with impaired T-helper cell function. The study thereby demonstrates that therapeutic cancer vaccination with **fima VACC** can be effective independent of T-helper cell functionality.

CLINICAL PROOF-OF-CONCEPT

▶ Phase I study in healthy volunteers with enhanced immune responses

▶ **Overall objective:**

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

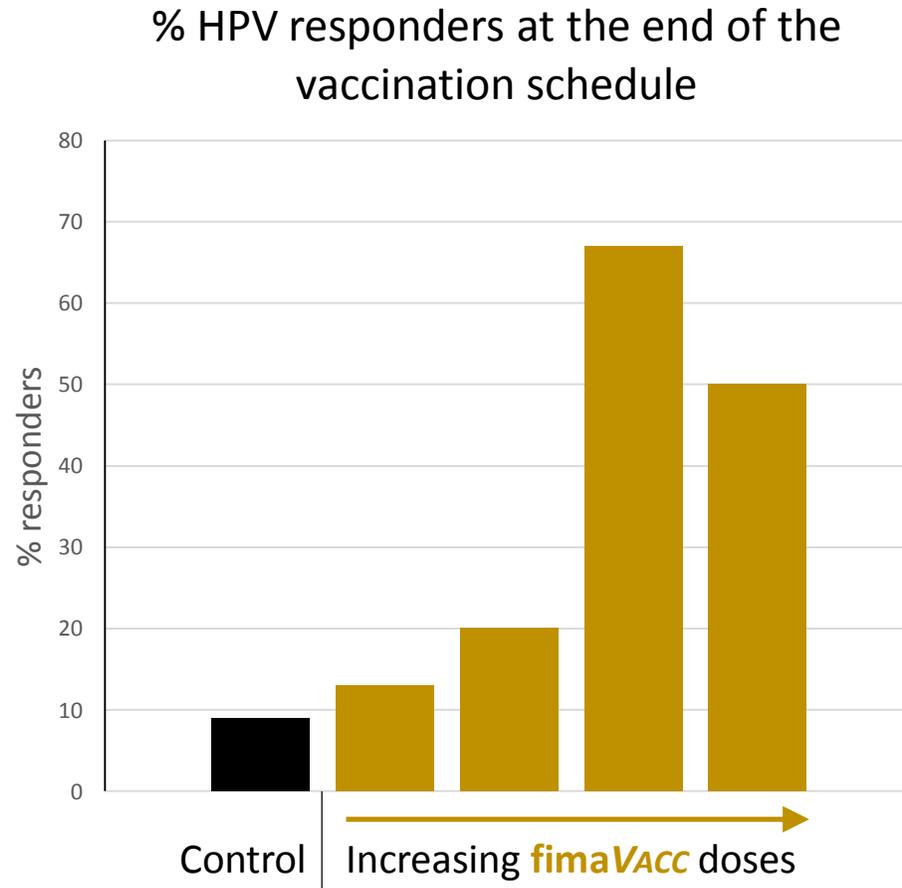
▶ **Results – compared to control **fima VACC** induces:**

- Substantial increase in number of T-cell responders to HPV E7 peptides
- Clearly enhanced overall T-cell responses
- More robust CD8 T-cell responses (notoriously difficult to induce with E7)
- Increased functionality of the induced CD8 T-cells

➤ **Highly sought-after features – especially for therapeutic vaccination**

OVERALL T-CELL RESPONSES – HPV E7 PEPTIDES

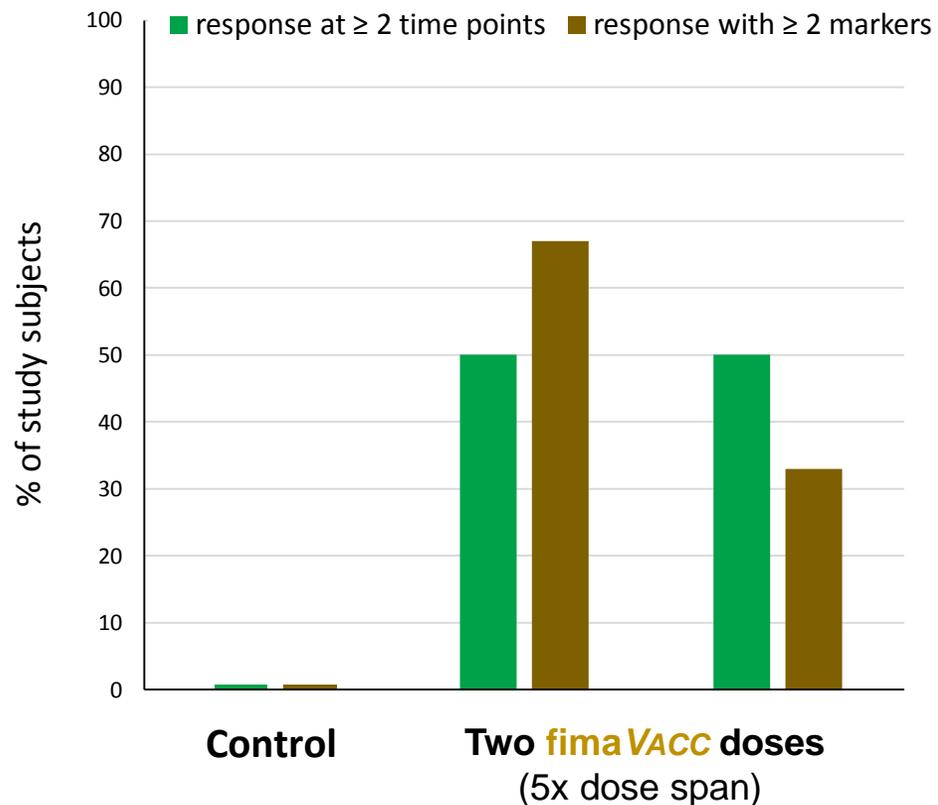
- ▶ Substantial increase in the percentage of subjects responding to vaccination



fima VACC induces more overall T-cell (CD4 + CD8) responders than the control with a state of the art adjuvant technology (Hiltonol), after completion of the HPV E7 vaccination schedule

CD8 T-CELL RESPONSES – HPV E7 PEPTIDES

▶ **fima VACC** induces more robust CD8 responses with polyfunctional CD8 T-cells



- **fima VACC** induces more CD8 T-cell responders and more robust responses across a wide tolerable dose span
- CD8 T-cell polyfunctionality indicates the ability of the T-cells to combat cancer cells and give protection against viral infections
- Flow cytometry analyses by group of the new SAC* member Prof. Sjoerd van der Burg at Leiden University Medical Center

*SAC: Scientific Advisory Committee

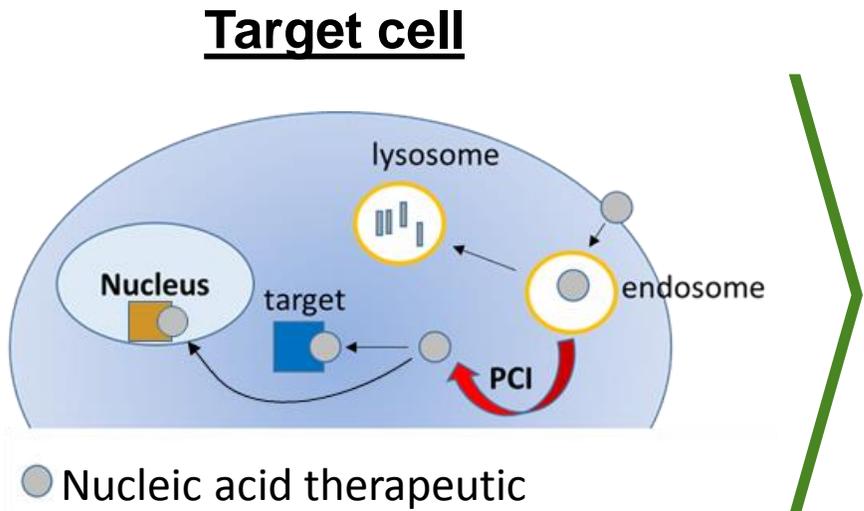
SOLID PROGRESS OF THE **fima VACC** PROGRAMME

▶ Successful clinical proof-of-concept

- ▶ The Phase I study provides successful clinical proof-of-concept for **fima VACC**
 - Proof of concept and efficacy in terms of intradermal dosing in humans
 - A positive overall characterisation of tolerability, with efficacy seen across a wide tolerable dose span
- ▶ Assessing the format for publication and presentation of the study results
- ▶ Strategy for **fima VACC** is two-pronged; utilising the Phase I results in direct partnering efforts and plan for clinical proof-of-concept in a disease setting

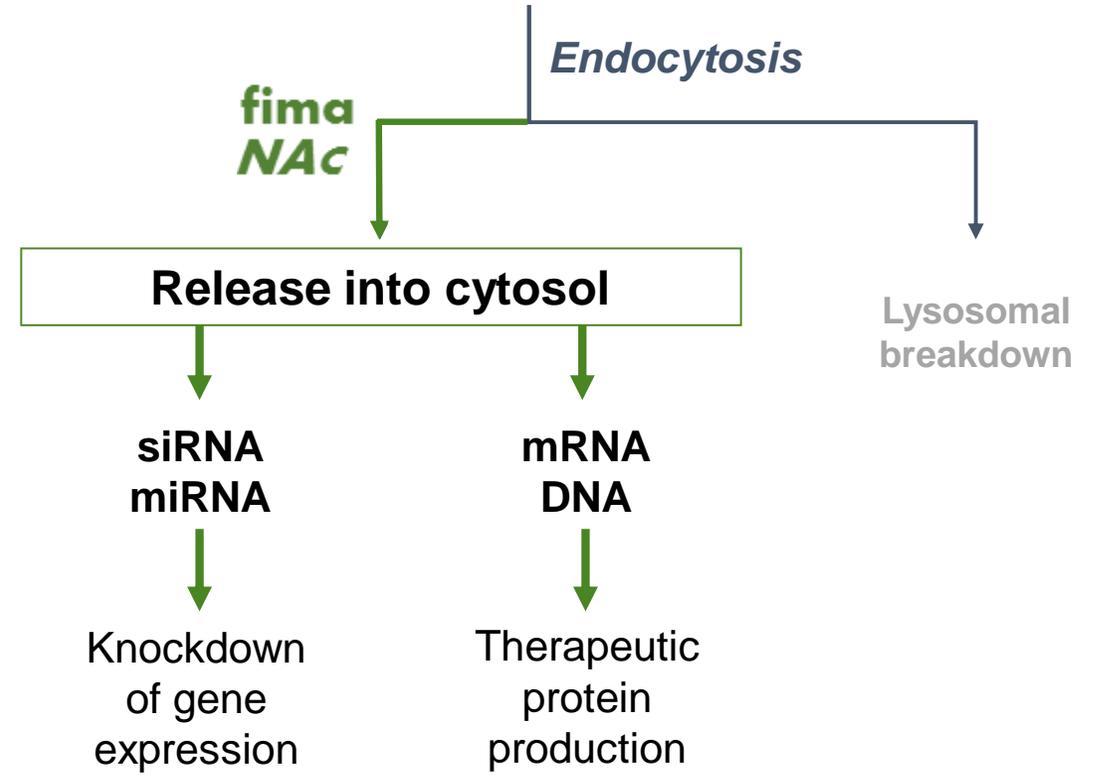
PCI TECHNOLOGY

► **fimaNAC** – mode of action



E.g.: {

Nucleic acid therapeutic



PROMISING INITIAL RESPONSE ON PATENT APPLICATION

- ▶ Patent application on mRNA delivery with **fimaNAc**

- ▶ The International Search Authority has provided a positive International Search Report on an mRNA delivery patent
- ▶ May give valuable intellectual property for **fimaNAc** mRNA delivery until 2039
- ▶ mRNA therapeutics is an emerging field with massive investments and broad potential applicability
- ▶ Sufficient intracellular delivery remains a major hurdle to realise the potential
- ▶ The majority of **fimaNAc** collaborations concern mRNA therapeutics

RESEARCH COLLABORATIONS

▶ Six collaborations established with key players in nucleic acid therapeutics

▶ Top-10 large Pharma collaboration extended to end of 2019 to complete the *in vivo* research under this agreement, with an additional 6 months period for determination of potential next steps

fimaNAC

Top-10
large
pharma



FINANCE

▶ Key financial figures

- ▶ Other income (public grants) in line with previous year
- ▶ Operating result impacted by planned start-up activities and initiation of the RELEASE study

(figures in NOK 1,000)	Q2 2019	Q2 2018	1H 2019	1H 2018	FY 2018
Other income	2,425	2,137	4,850	4,375	9,585
Operating results	-27,050	-7,193	-44,979	-21,855	-44,519

(figures in NOK 1,000)	Q2 2019	Q2 2018	1H 2019	1H 2018	FY 2018
Net change cash and cash equivalents	-27,137*	-10,181	-47,705**	-22,384	298,537***
Cash and cash equivalents	301,621	28,405	301,621	28,405	349,326

Including effects from exchange rate fluctuation on bank deposits in EURO

*Q2 2019 effect of NOK 595

**1H 2019 effect of NOK -4,850

***FY2018 effect of NOK 9,092

STRENGTHENING THE ORGANISATION

- ▶ SAC and BoD has been strengthened with prospectively important expertise

- ▶ The Scientific Advisory Committee has been further strengthened with immunological expertise by the appointment of Professor Sjoerd van der Burg, to ensure adequate scientific support to the **fima VACC** programme
- ▶ The Board of Directors has by the appointment of Mrs Hilde Furberg been further strengthened with commercial experience and expertise, important for the future development of the company

KEY ACHIEVEMENTS & NEAR-TERM MILESTONES

- | | | |
|---------|-------------------|--|
| 2H 2018 | ✓ fimaCHEM | Design of pivotal study finalised |
| 2H 2018 | ✓ fimaCHEM | Preliminary safety of repeated treatment reported |
| 1H 2019 | ✓ fimaVACC | Completion of Phase I immune analyses |
| 1H 2019 | ✓ fimaCHEM | Safety of repeated treatment confirmed |
| 1H 2019 | ✓ fimaCHEM | First patient enrolled in pivotal bile duct cancer study |
| 2H 2019 | ➤ fimaCHEM | First US patient enrolled in pivotal bile duct cancer study |
| 2H 2019 | ➤ fimaVACC | Phase I results published and presented at key conference |
| 2020 | ➤ fimaCHEM | First Asian patient enrolled in pivotal bile duct cancer study |

INVESTMENT HIGHLIGHTS

Market

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

Lead product

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**

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

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