



PCI BIOTECH

Unlocking the potential of innovative medicines

Full year 2017 PRESENTATION

March 20, 2018

Per Walday, CEO

Ronny Skuggedal, CFO



PCI BIOTECH

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HIGHLIGHTS

► 2017 – a year of significant achievements

fima *CHEM*

- Received important regulatory guidance on requirements to a pivotal bile duct cancer study
 - Granted US Orphan Drug Designation for fimaporfin in bile duct cancer by FDA
 - Encouraging interim overall survival data from Phase I in bile duct cancer
-

fima *VACC*

- Promising interim clinical results suggesting high rates of enhanced and early T-cell responses at tolerable dose levels
 - Awarded up to NOK 14.3 million from the Norwegian Research Foundation
-

fima *NAC*

- Progress in research collaborations with key players, with the top-10 pharma collaboration expanded and extended twice
-










Corporate

- Completion of a fully underwritten rights issue of NOK 70 million
- Strengthened management team further by appointment of Dr Hans Olivecrona as CMO
- Initiated process for transfer of listing from Oslo Axess to Oslo Børs (subsequent event)

PCI BIOTECH AT A GLANCE

▶ Unlocking the potential of innovative medicines

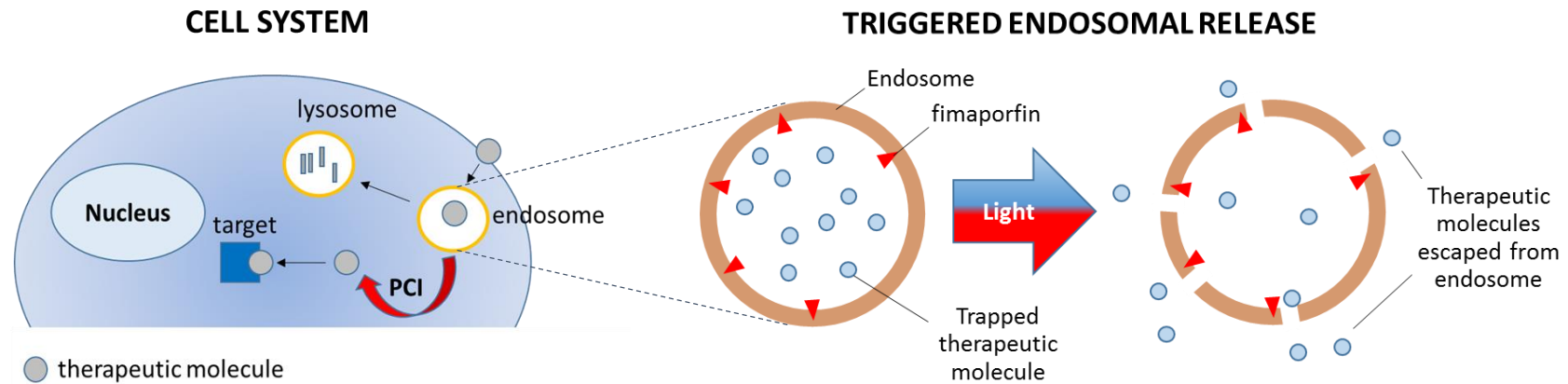
- ▶ Listed on Oslo Axess (PCIB:NO)
- ▶ Photochemical internalisation (“PCI”) technology, originating from the Norwegian Radium Hospital

Programme	Indications / Therapeutics	Preclinical	Phase I	Phase II	Status
	 <i>Bile duct cancer / gemcitabine</i>				Preparing for pivotal study 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

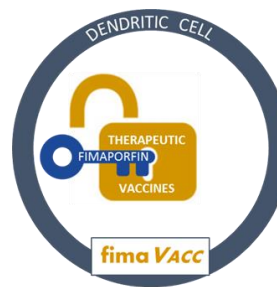
► Enabling drugs to reach intracellular therapeutic targets



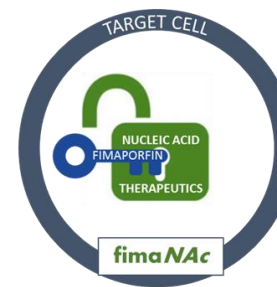
PCI – the solution to a key challenge for several modalities



Enabling approved drugs to fulfil unmet local treatment need



Enhancing cellular immune responses important for therapeutic effect

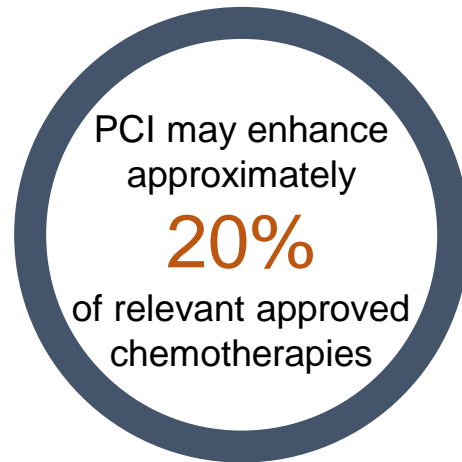


Providing a delivery solution for nucleic acid therapeutics

THE SOLUTION TO A KEY CHALLENGE

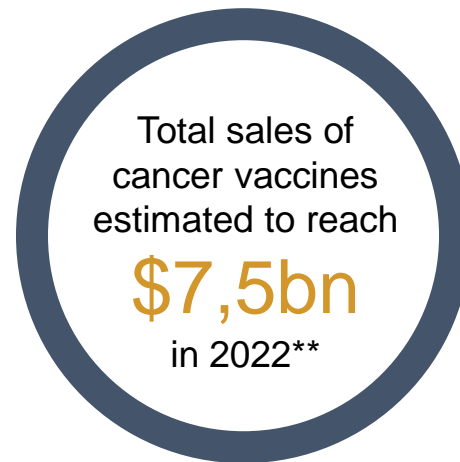
▶ Three well-defined development programmes

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

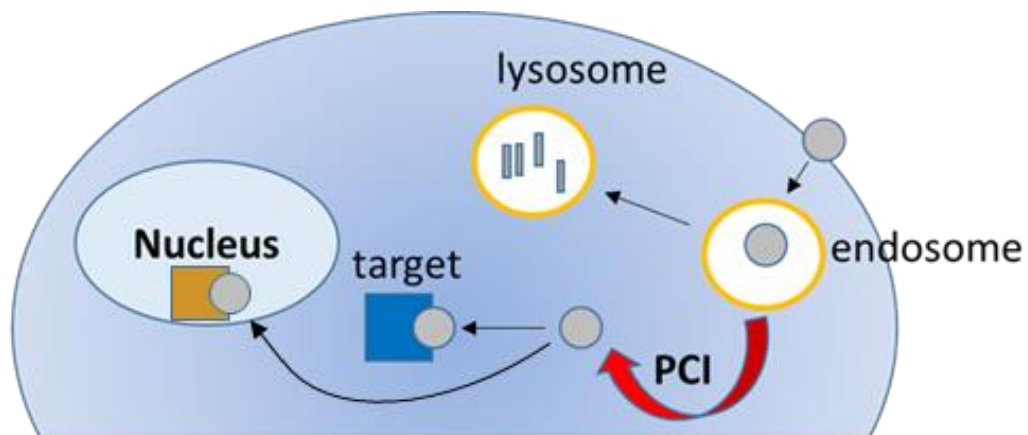
** GBI Research (2016) Global Cancer Vaccines Market to 2022

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

PCI TECHNOLOGY

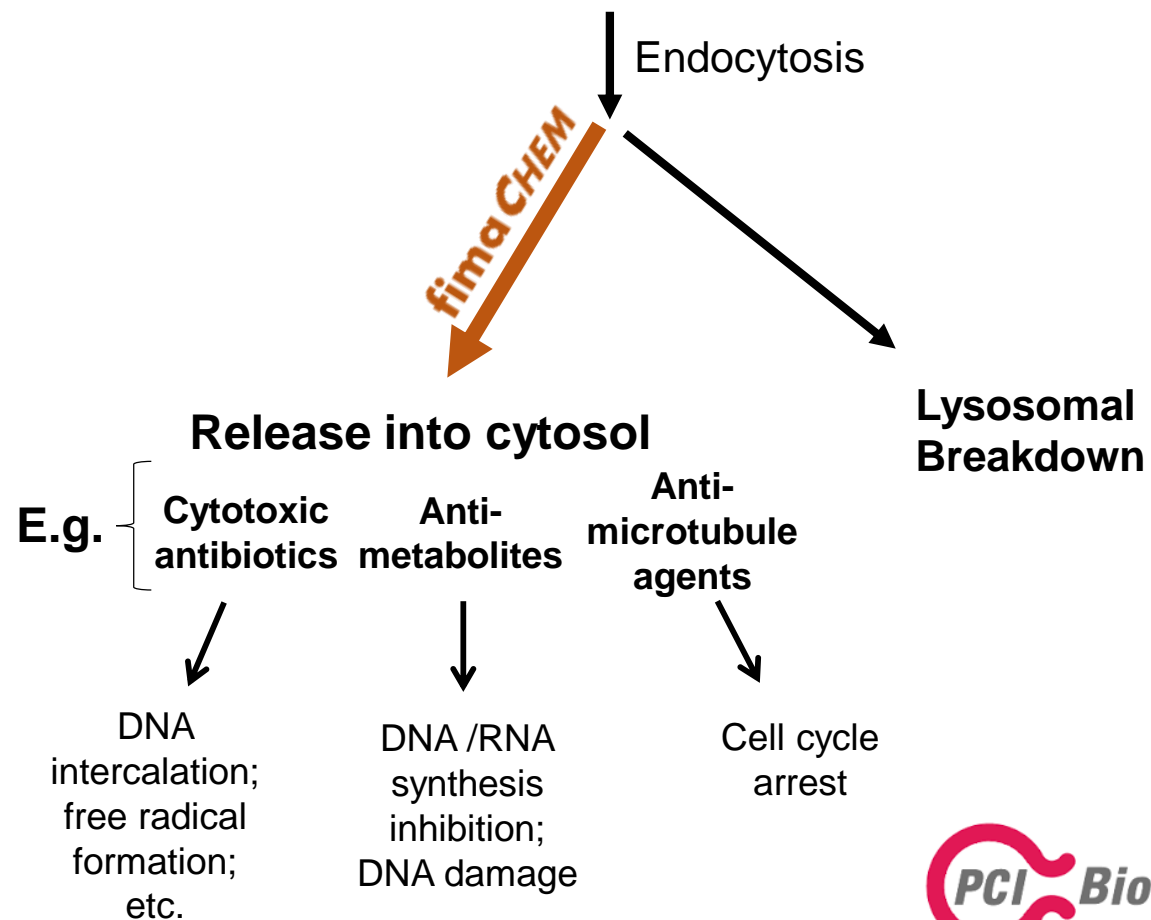
► fimaCHEM – mode of action

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

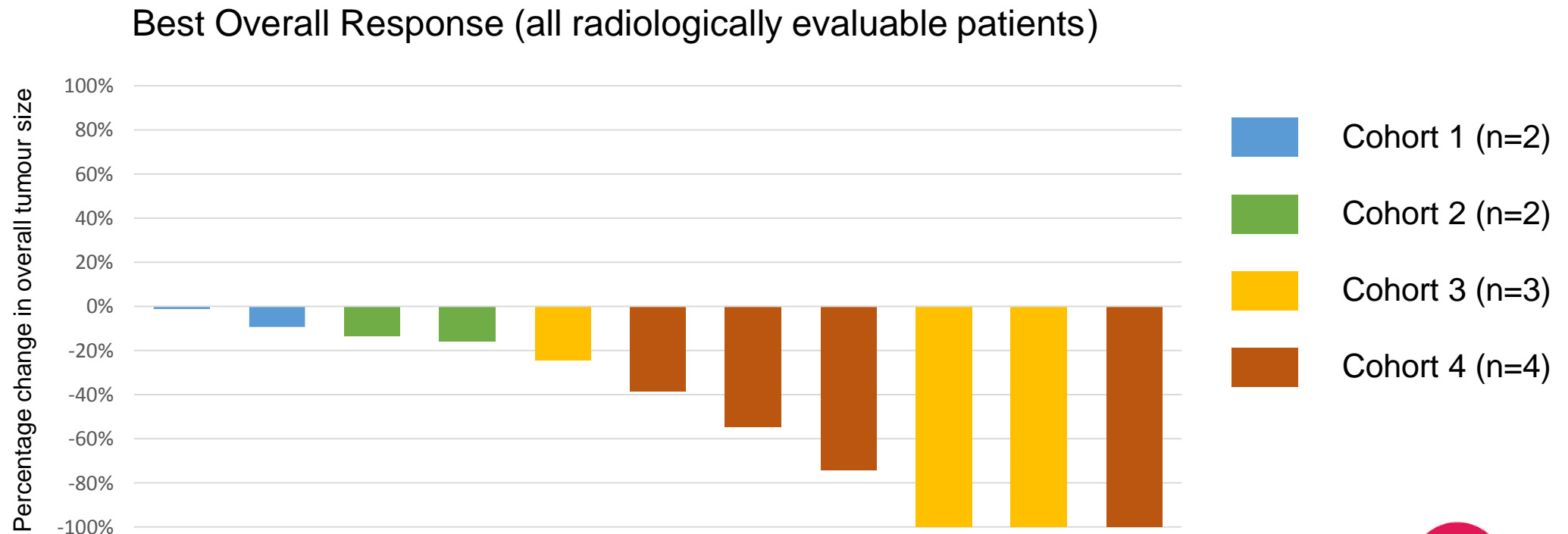
Inducing immunogenic tumour cell death

- 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

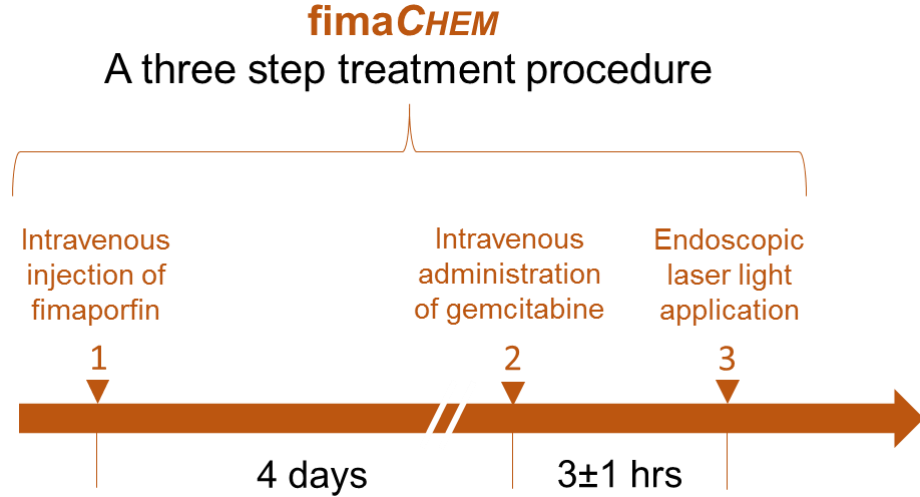
► Encouraging early signs of efficacy in Phase I

- Interim average overall survival (OS) of all 16 patients in Phase I was 16.8 months per December 2017, with 25% of the patients still being alive. Median OS ended at 14.4 months.

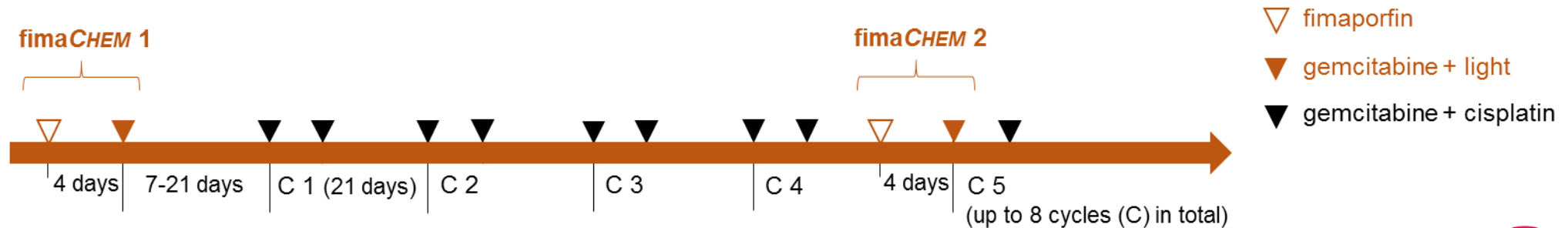


BILE DUCT CANCER – PHASE I EXTENSION STUDY

▶ Repeating the **fimaCHEM** treatment with the aim to further enhance efficacy



- ▶ Exploring safety of repeating the **fimaCHEM** treatment in an extension to Phase I
- ▶ Intention is to perform the pivotal study with repeated treatment
- ▶ The study is done in parallel with other preparations for the next phase



INOPERABLE EXTRAHEPATIC BILE DUCT CANCER

▶ Status and strategy going forward

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

▶ Initiation of pivotal study moved to 2H 2018

- Pending regulatory clarifications on the interim analysis opportunity has held up pivotal study preparations
- Four patients included in the Phase I extension study in 2017 – complete safety read-out expected 2H 2018

COMPETITIVE LANDSCAPE

- ▶ A limited pipeline targeting different inoperable cholangiocarcinoma (CCA) indications

Estimated number of competitor products in development in different CCA indications¹

Target	Phase I/II		Pivotal Phase	
	First line	Second line	First line	Second line
All CCA	7	5	1 ^{***}	1
iCCA* only	-	2	-	2
eCCA** only	-	-	-	-

* Intrahepatic cholangiocarcinoma

** Extrahepatic cholangiocarcinoma – the target population for fimaCHEM

*** Announced that a pivotal study will be initiated in 2018 (also included under Phase I/II)

- ▶ Phase I are often “basket studies”, where CCA may be one of several indications
- ▶ Several CCA studies are Investigator initiated / sponsored by academia
- ▶ Only one pharmaceutical treatment – fimaCHEM – offers a local boost in the bile duct
- ▶ Localised non-specific treatments are on the market (RFA² and radiation), but with limited documented benefit

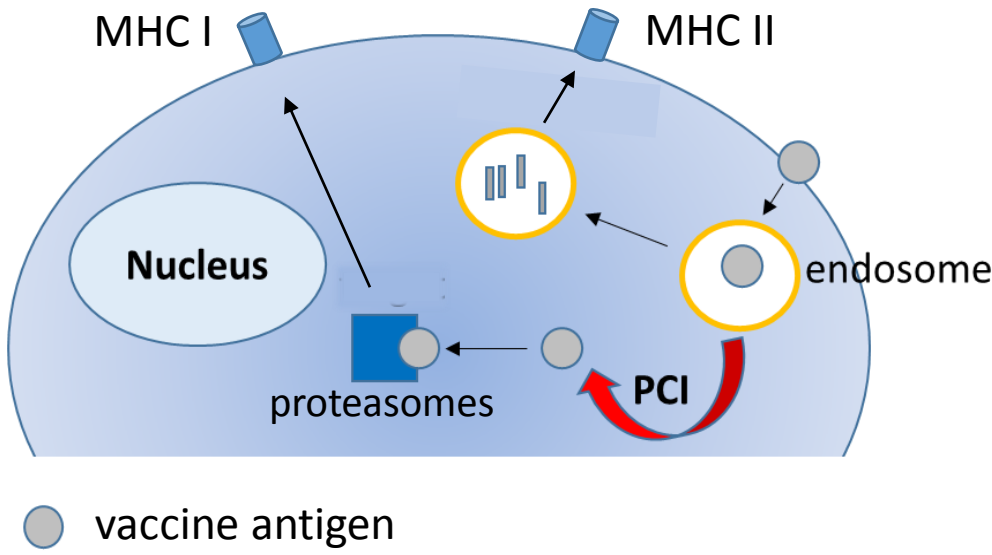
¹ Internal PCI Biotech analysis

² Radiofrequency ablation

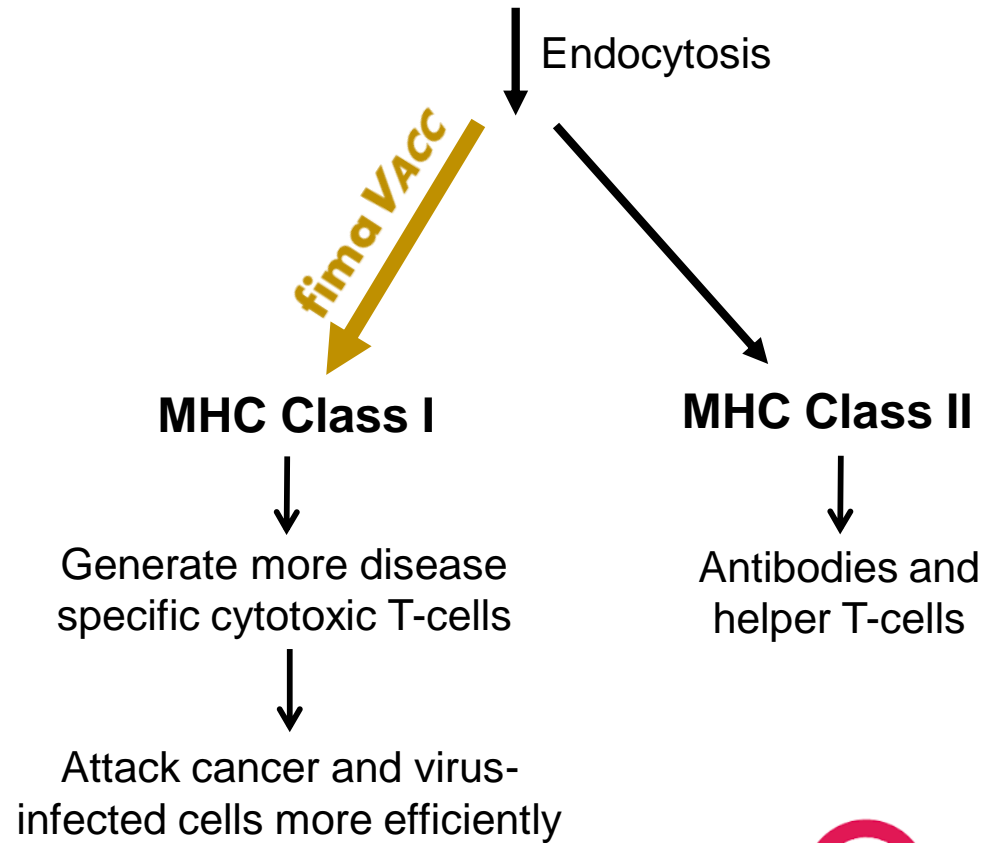
PCI TECHNOLOGY

► fima VACC – mode of action

Dendritic cell



Vaccine



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

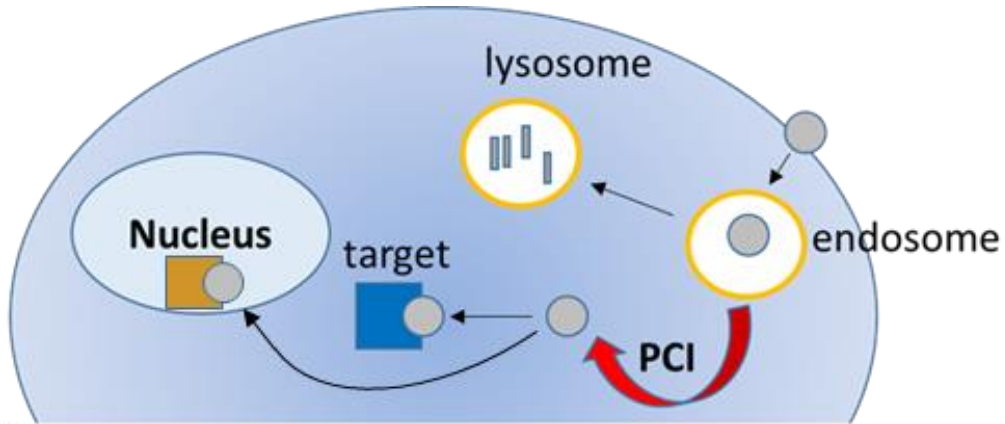
▶ Status:

- More than 90 subjects have been included to date
- 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 seen at the lowest fimaporfin dose → study expanded – lower doses currently being investigated
- Part 3 TBD
- Expected completion: 2H 2018

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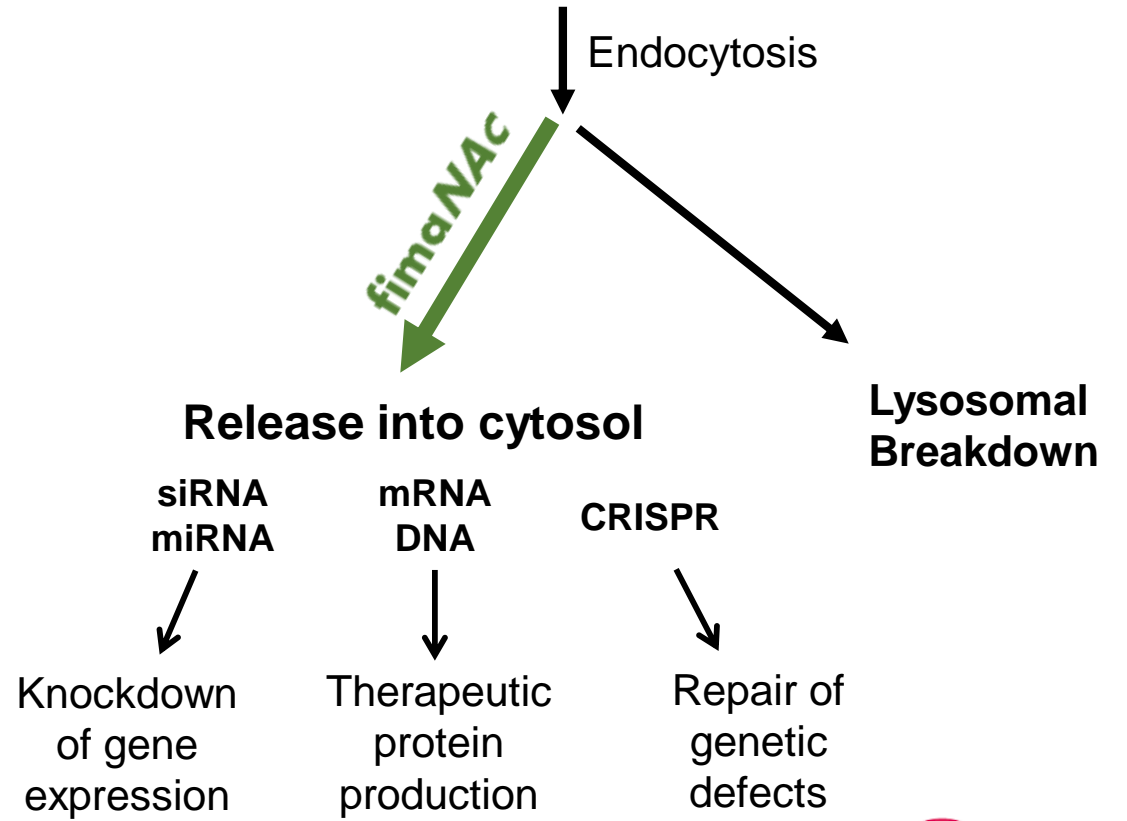
► **fimaNAC** – mode of action

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 MirlImmune acquisition

Top-10 large pharma

- Initiated Q3 2015. A global leader in nucleic acid therapeutics
- Expanded to include *in vivo* studies – current agreement extended to end of Q2 2018



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

(in NOK 1,000)	2017	2016
Other income	10 250	10 475
Operating results	-43 431	-33 027

(in NOK 1,000)	2017	2016
Cash	50 789	14 002

(in NOK 1,000)	2017	2016
Cash flow operating activities	-30 620	-35 693

- More than NOK 10 million in public grants
- Operating result impacted by shared based payment, without cash effect
- Cash position to cover preparations for pivotal phase
- Initiated a process for transferring the listing from Oslo Axess to Oslo Børs

GOOD PROGRESS IN ALL AREAS

▶ 2017 – a transformative year

- ✓ **fimaCHEM** Regulatory clarity on fastest route to market
- ✓ **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 further with Dr Olivecrona as CMO

KEY MILESTONES ANTICIPATED

▶ Through 2018

- 1H 2018 ▶ **Corporate** Transfer of listing from Oslo Axess to Oslo Børs
- 2H 2018 ▶ **fimaCHEM** Safety of repeated treatment
- 2H 2018 ▶ **fimaCHEM** Initiation of pivotal bile duct cancer study
- 2H 2018 ▶ **fimaVACC** Phase I in healthy volunteers completed

PCI BIOTECH HOLDING ASA

► Enquiries

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