BIOEurope Spring 2016

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Per Walday, CEO

PCI Biotech

PCI BIOTECH

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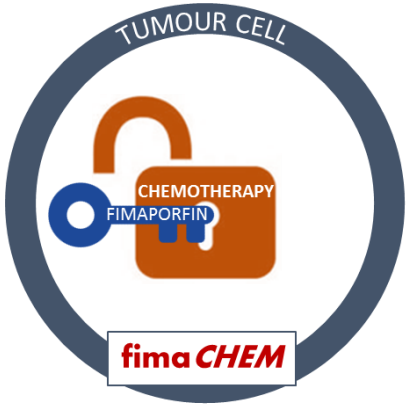
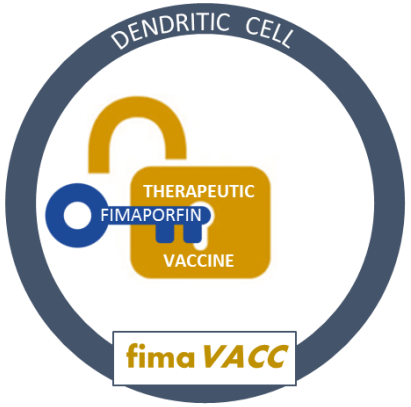
PCI BIOTECH AT A GLANCE

▶ Unlocking the potential of innovative medicines

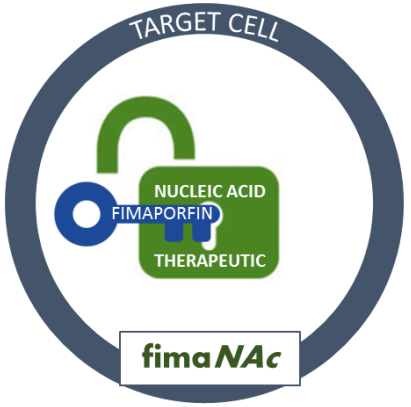
- A listed (PCIB:NO) cancer-focused biotech company
- Photochemical internalisation (“PCI”) technology, originating from the Norwegian Radium Hospital
- Clinical program
  - **fima*CHEM*** – Phase I/II with fimaporfin (Amphinex®) for the orphan indication inoperable bile duct cancer
- Pre-clinical programs
  - **fima*VACC*** – Vaccination technology that provides strongly enhanced T-cell responses
  - **fima*NAc*** – Efficient intracellular delivery of nucleic acid therapeutics



Bile duct cancer study with promising early signs of efficacy in Phase I and Phase II about to start



Proprietary vaccination technology moving towards clinical validation, and one active research collaboration



Preclinical program with two active research collaborations, one with top tier pharma

PHOTOCHEMICAL INTERNALISATION

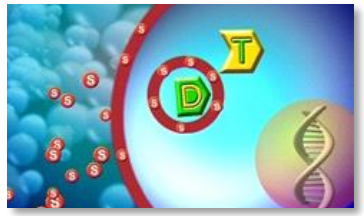
► Triggered endosomal release through illumination

STEP 1:

- Fimaporfin (S) and the active molecule (D) are injected into the body and reaches the target cells

STEP 2:

- Fimaporfin (S) and the active molecule (D) are taken up by the cell, but D is unable to reach the target (T), as it is encapsulated in an endosome
- S is washed away from the cell membrane, but trapped in endosomes



STEP 3:

- Light activates fimaporfin (S) in the membrane of the endosome
- The membrane integrity is affected and the active molecule released

STEP 4:

- The active molecule (D) can now bind to its target (T) and initiate the therapeutic response

D

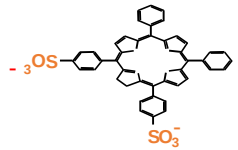
The active molecule

- Anticancer agent, e.g. bleomycin, gemcitabine
- Oligonucleotide, e.g. siRNA
- Protein, e.g. antibody-drug conjugate
- Peptide: e.g. antigen

S

The PCI component

- Light sensitive component
- Fimaporfin - Amphinex®



T

The target

- Target for the active molecule
- E.g. DNA, mRNA, enzyme, microtubuli

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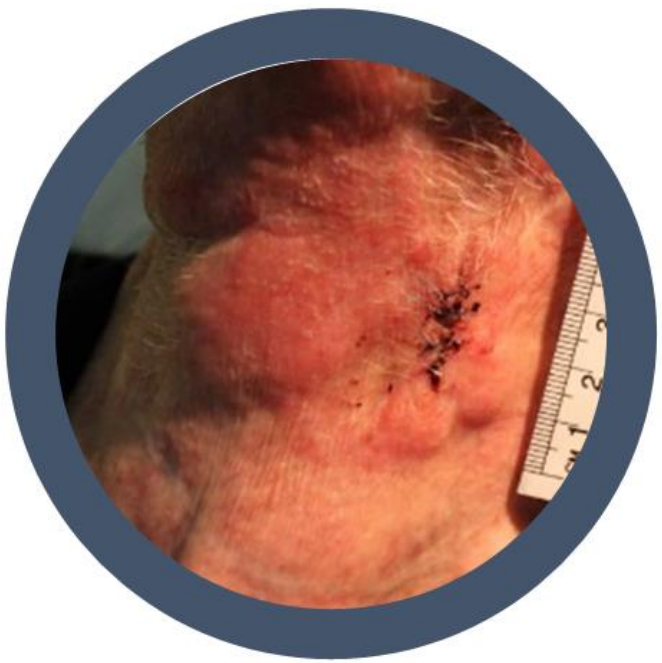
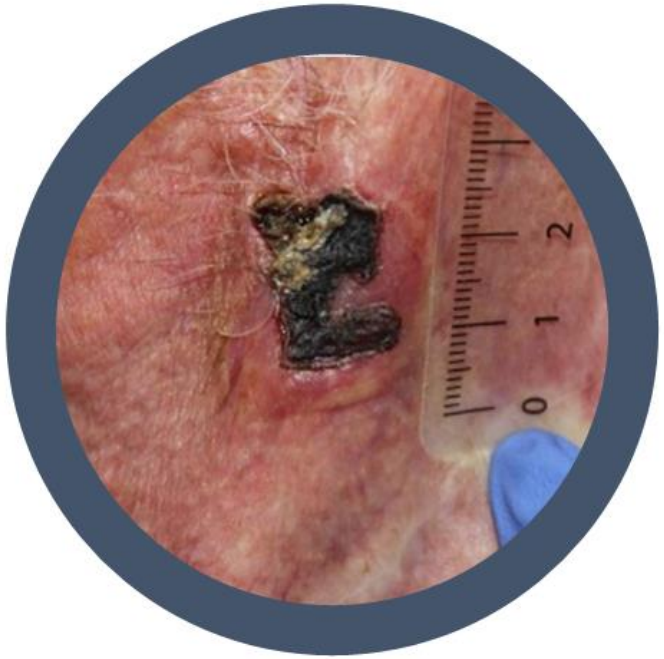
Clinical Technology Validation

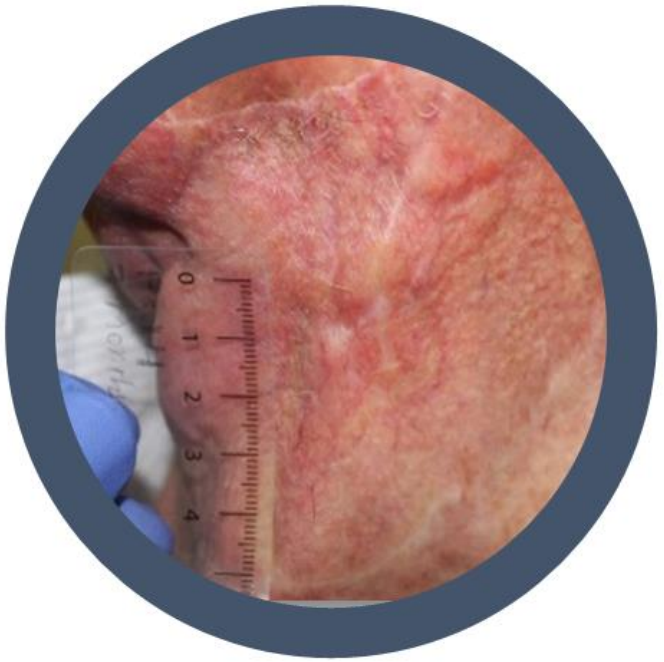
- Phase I of fimaporfin – combined with bleomycin in recurrent/metastatic cancer



Baseline



Day 28



Day 90

- Very promising early signs of tumour response across a range of fimaporfin dose levels
- Apparent strong selectivity for cancer in several patients
- Well tolerated with appropriate pain control and anaesthesia

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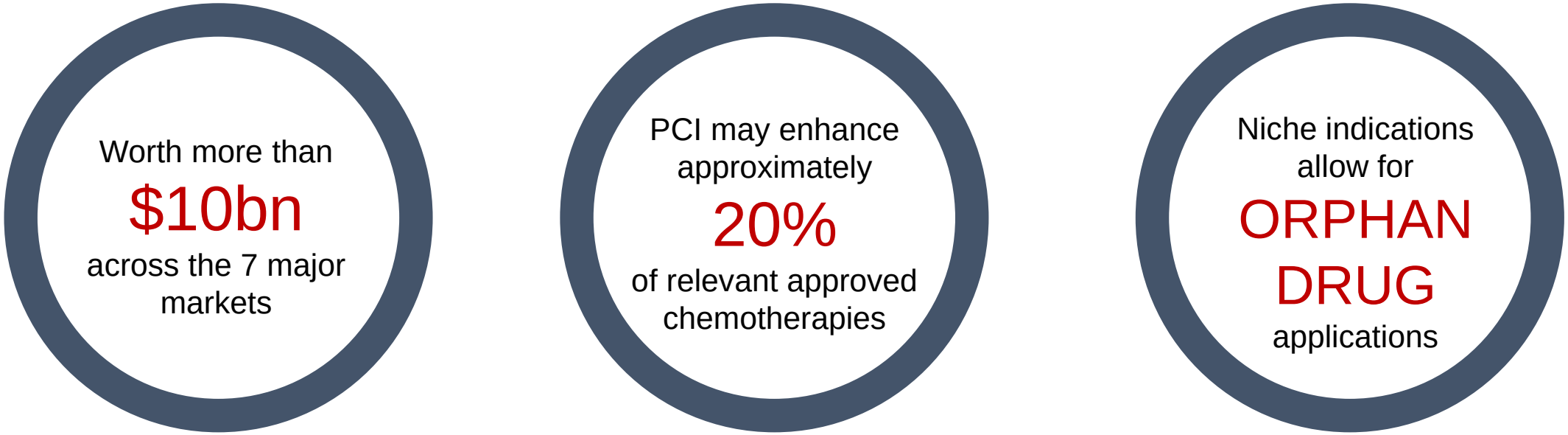
CHEMOTHERAPEUTICS

- A cornerstone in current cancer therapy



- fimaCHEM may enable approved drugs to fulfil unmet local treatment needs
- Aim is to complete Phase II in cholangiocarcinoma before out-licensing
- Opportunity for development in further niche indications

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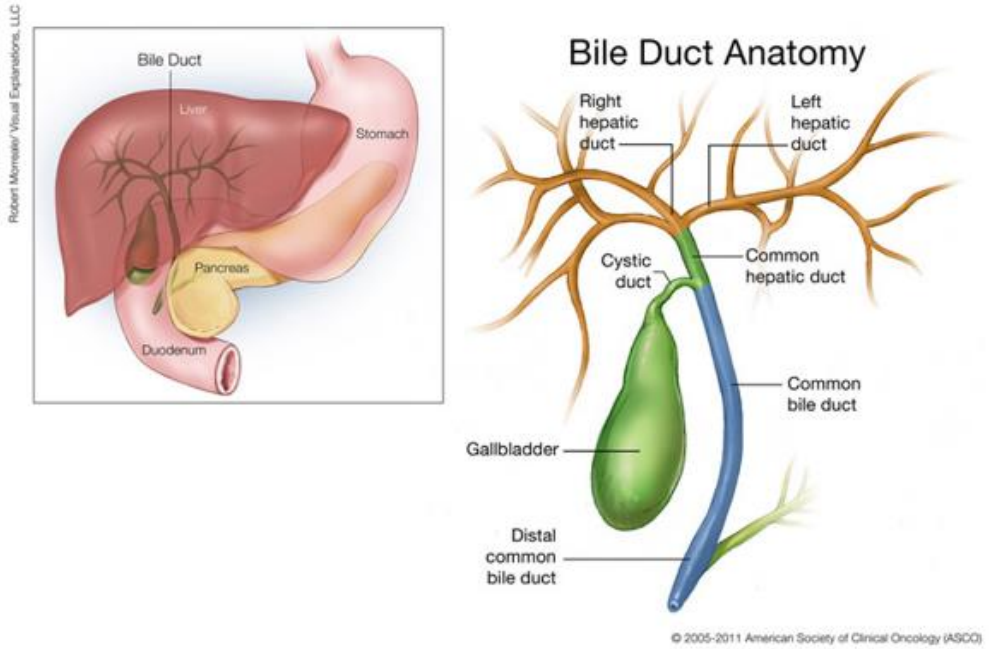
BILE DUCT CANCER

- A rare but fatal disease



- **Five year survival less than 5%**
- **Remarkable resistance to chemotherapy**
- **Estimated market potential of up to USD 500m for efficacious treatment**
- **Phase I/II trial ongoing with fimaprofin**
  - combination with gemcitabine
  - open-label, multi-center trial in up to 45 patients
  - activation of fimaporfin by intraluminal illumination

BILE DUCT CANCER

- Progressing into Phase II with promising early signs of efficacy

Why target bile duct cancer?

- Significant inoperable patient population with high unmet local treatment need
- No approved medical treatments
- Limited development pipeline
- Active chemotherapy enhanced by PCI
- Easy access with light through routine endoscopic methods

Attractive due to orphan benefits and absence of satisfying treatments

Current status and plans

- Safety driven European Phase Ib completed
- Fourth dose cohort concluded Jan 2016 – no safety concerns
- Promising early signs of efficacy in third dose cohort – awaiting fourth dose cohort results
- Progressing into Phase II
- Increasing number of sites
- Opening of IND and including US sites
- Obtain orphan designation

BILE DUCT CANCER – CLINICAL PHASE I/II STUDY

- Preliminary response data

- **6 months radiology (CT) data from 3 dose cohorts**

	PD	SD	PR	CR	NA*
Cohort 1	1	1			1
Cohort 2		1			2**
Cohort 3		1	1	1	
Cohort 4	Not yet available – subjects on-going				

* Not measurable / Not evaluable by CT
** Considered SD at 6 months by the investigator

- Subjects are in the study for 6 months after PCI treatment
- Dose levels given in cohort 1 and 2 are below what is expected to be effective from previous clinical experience

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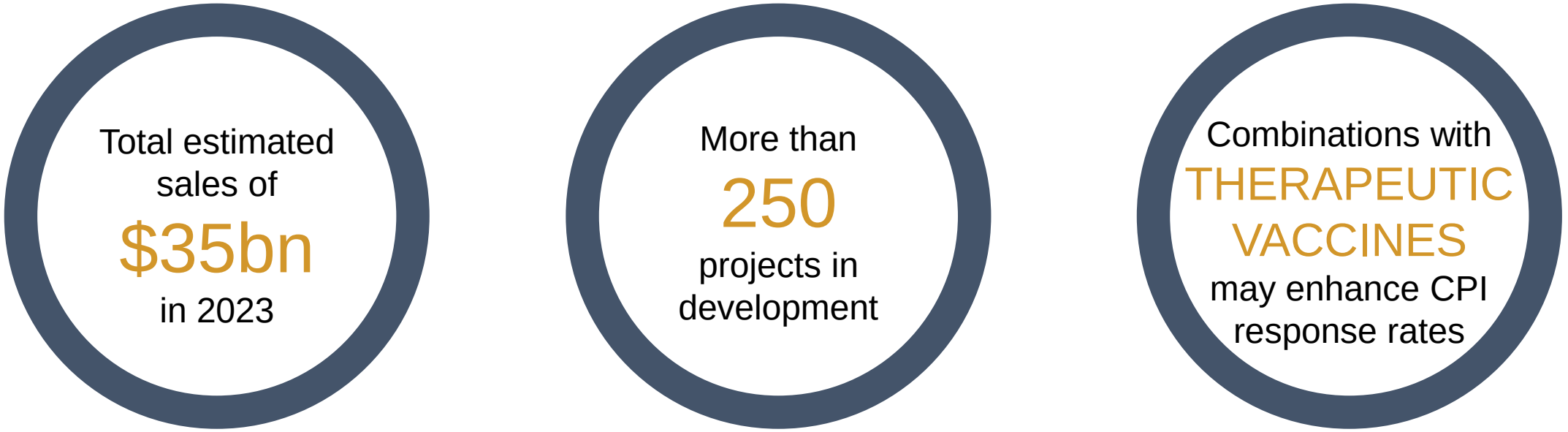
IMMUNOTHERAPY

- A new hope for millions of patients



- fima VACC enhances cellular immune responses important for therapeutic effects
- Moving towards clinical validation, potentially in healthy volunteers
- Aim is to out-license the technology on non-/semi-exclusive basis
- Opportunity to develop own therapeutic vaccination products

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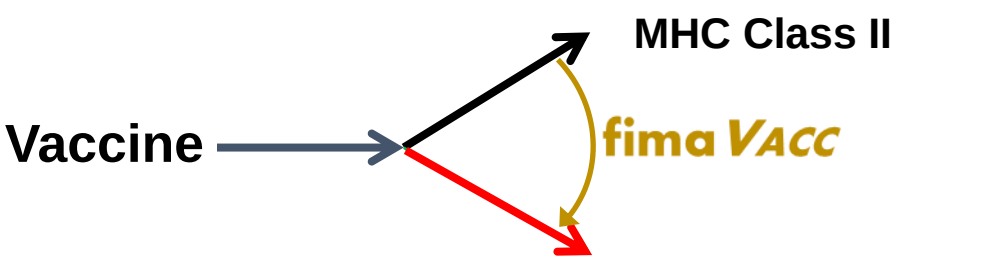
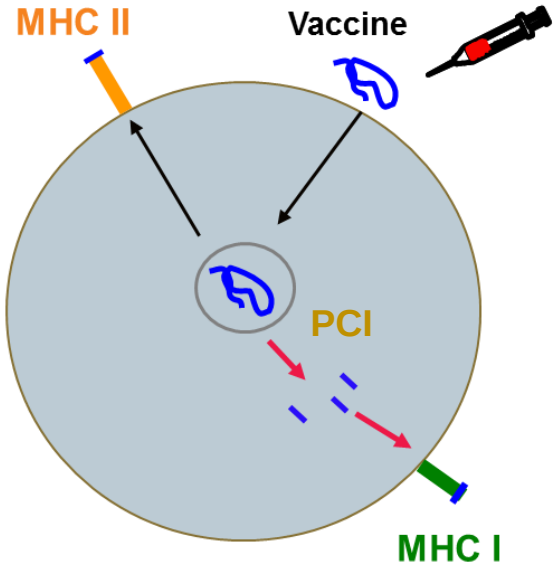
PCI FOR VACCINATION

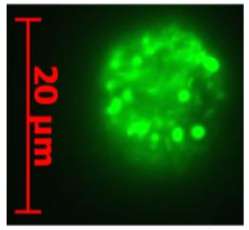
- Enhancing cytotoxic T-cell response by light-induced cross presentation



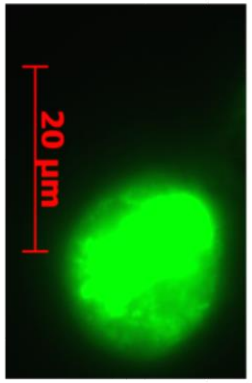


Generate more disease specific cytotoxic T-cells

Attack cancer and virus-infected cells more efficiently



Antigen in endosomes



After fima VACC treatment

Ovalbumin antigen in JAWSII dendritic cells

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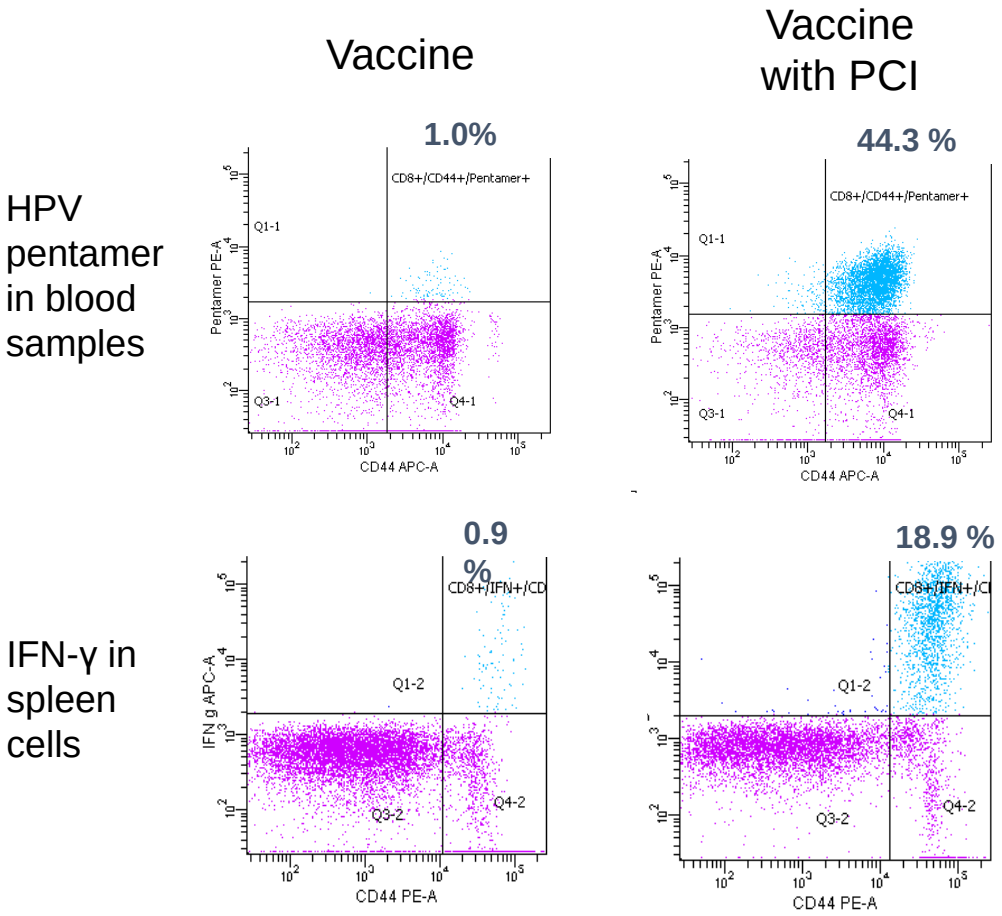
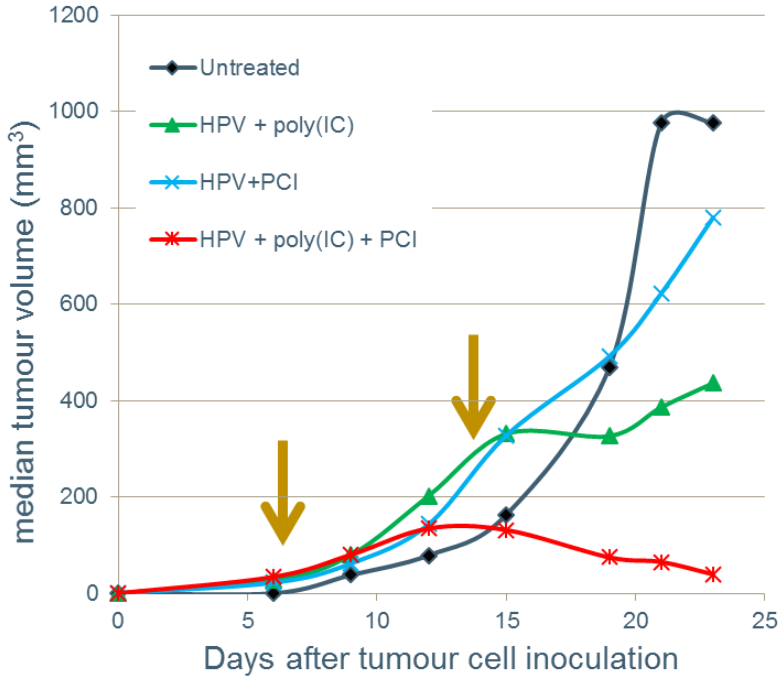
THERAPEUTIC VACCINATION

- *In vivo* immunisation with HPV long peptide

fima VACC strongly enhance CD8 T-cell response and induces strong anti-tumour response





- Intradermal vaccination at days 6 and 13 after tumour cell inoculation
- 5 animals per group

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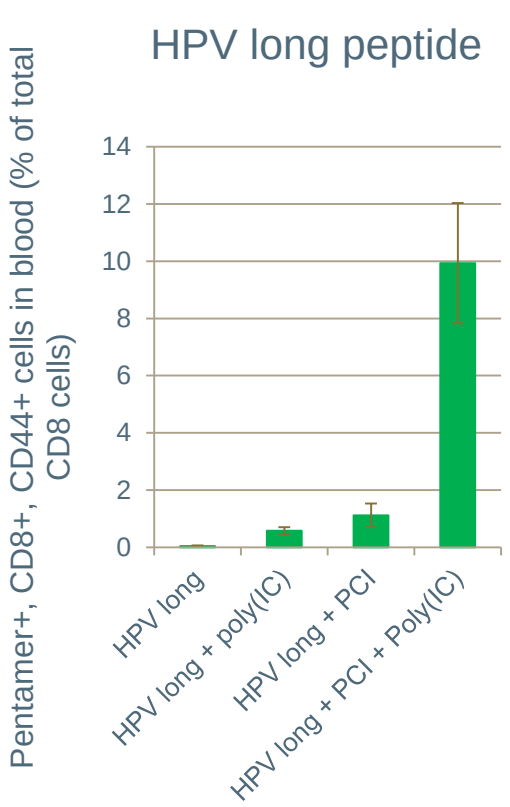
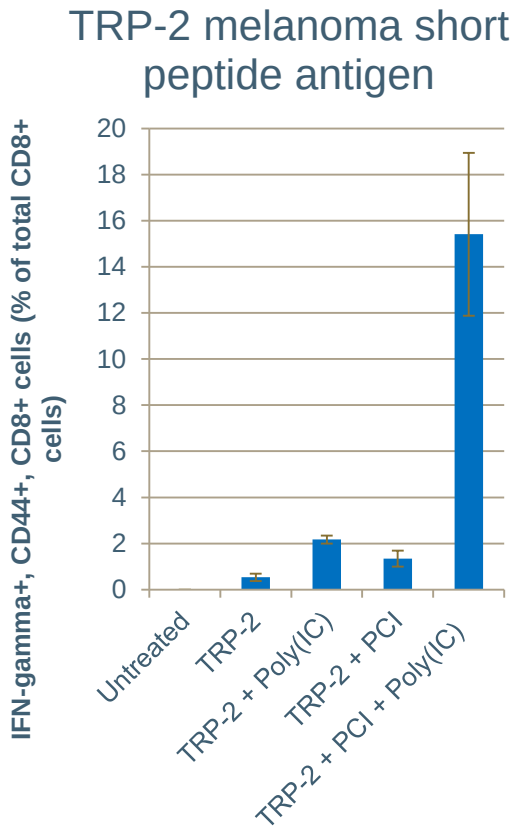
SYNERGY WITH OTHER TECHNOLOGIES

► Acts synergistically with other vaccination enhancement technologies

- Acts synergistically with several commonly used vaccine adjuvants
- Works with many different peptide antigens and stimulates both CTL proliferation and IFN-γ production





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THERAPEUTIC VACCINATION WITH fima VACC

- 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
  - particulate antigen formulations
  - prophylactic & therapeutic vaccination
- Safety of fimaporfin confirmed in Phase I studies
- Excellent stability
  - stable at room temperature
  - stable in solution
  - can be autoclaved
- Cost effective synthesis

NUCLEIC ACID THERAPEUTICS

- A treatment modality with huge potential

- fimaNAc may provide a delivery solution for many nucleic acid therapy applications
- Opportunistic collaborative approach
- Aim is to out-license the technology on non-/semi-exclusive basis

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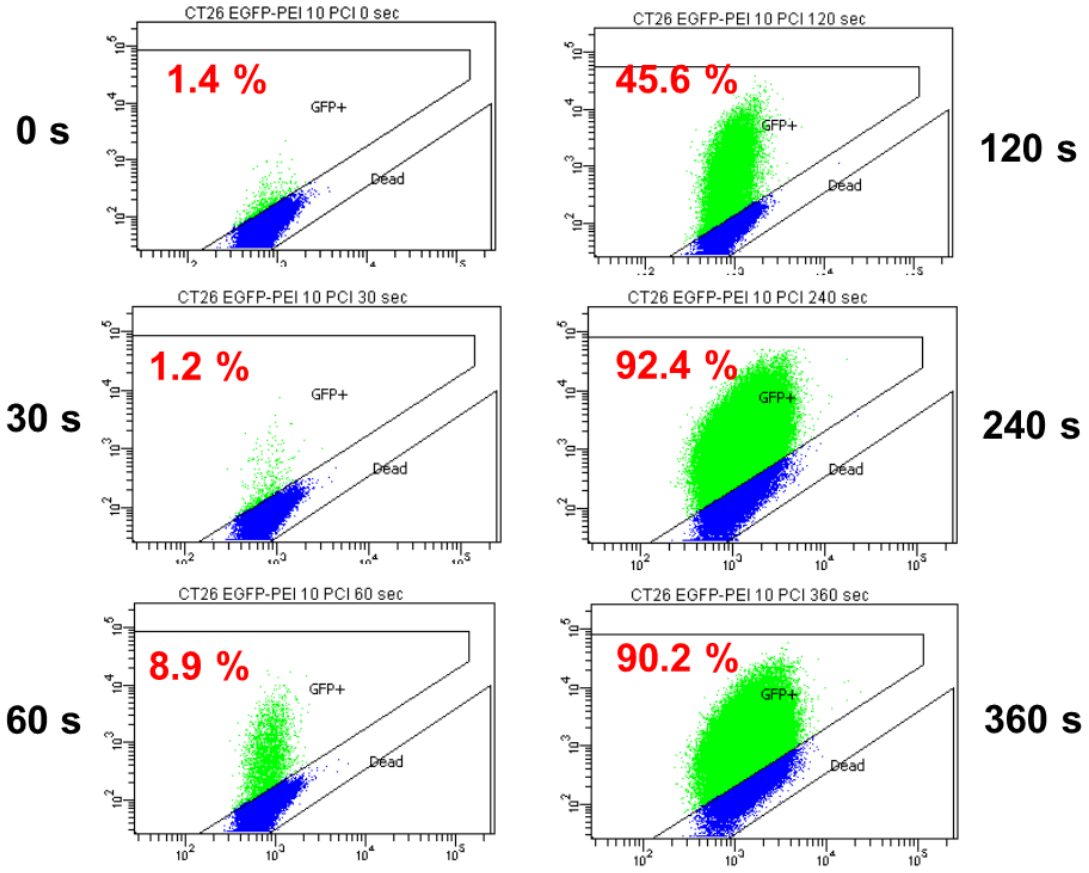
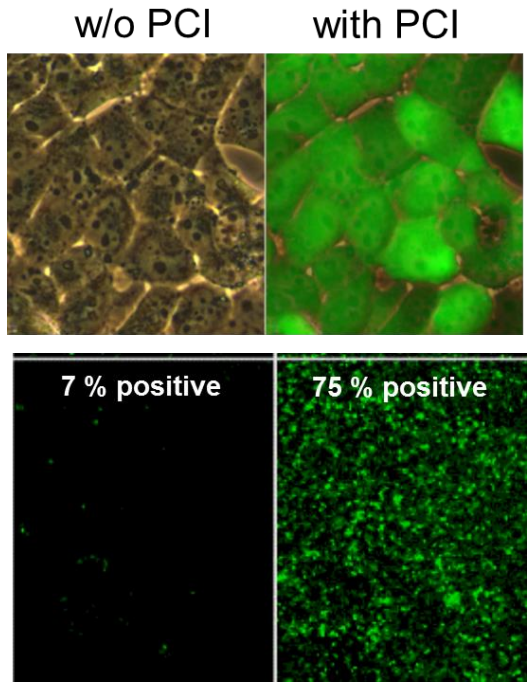
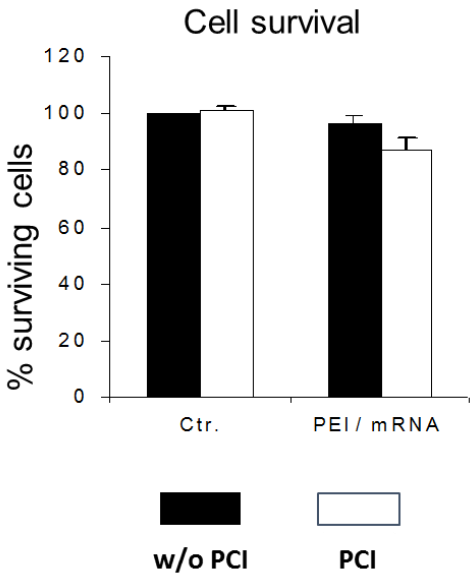
ENHANCING mRNA DELIVERY

- Strongly increased GFP synthesis with increasing light doses

fimaNAc with polyethylenimine vehicle







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Research Collaborations

- Three active collaborations within nucleic acid therapeutics and vaccination

Top-10 large pharma company

- Agreement signed in 3Q 2015
- Evaluate synergistic effects between companies' technologies
- One of the global leaders in nucleic acid therapeutics
- Collaborative research funded and initiated
- Data generated in research collaboration to be evaluated- potential for a further partnership

RXi Pharmaceuticals

- Agreement signed 2Q 2015
- RXi Pharmaceuticals listed on Nasdaq (NASDAQ: RXII)
- Discovers and develops innovative therapeutics within dermatology and ophthalmology
- Results achieved from this research collaboration to be evaluated- potential for closer collaboration

Ultimovacs

- Agreement signed 1Q 2016
- Ultimovacs AS, Norwegian immunotherapy company
- Developing UV1, a therapeutic cancer vaccine directed against human telomerase
- Results from this research collaboration to be evaluated- potential for closer collaboration

KEY MILESTONES THROUGH 2018

- Unlocking the true potential of innovative medicine