

PCI Biotech press release 24<sup>th</sup> August 2018

Attachment

# BILE DUCT CANCER – CLINICAL PHASE I STUDY

► Cohort IV is selected dose for pivotal study – limited but promising data (per Aug 2018)

Parameters	Cohort IV (N=6) (0.25mg/kg)	Phase I – full study (N=16) (0.06-0.25mg/kg)
1) Average number gemcitabine/cisplatin cycles	<b>6.0</b> (range: 0 - 8)	<b>6.4</b> (range: 0 - 8)
2) Patients w/ radiologically measurable lesions	<b>5/6 (83%)</b>	<b>11/16 (69%)</b>
3) Average overall tumour size	<b>5.2 cm</b> (range: 2.1 - 7.8 cm)	<b>4.1 cm</b> (range: 1.5 - 7.8 cm)
4) Objective Response Rate (ORR)	<b>3/5 patients (60%)</b> (2 PR; 1 CR)	<b>4/12 patients (33%)</b> (2 PR; 2 CR)
5) Interim average Duration of Response (DoR)	<b>15.4 months</b> (range: 8.0 – 20.2 months)	<b>12.4 months</b> (range: 6.5 – 20.2 months)
6) Overall Survival (OS)	mOS: <b>19.4 months</b> interim avg OS: 19.0 months (33% alive)	mOS: <b>14.4 months</b> interim avg OS: 18,5 months (19% alive)

The table contains key background and measured endpoints for the Phase I dose-escalation study. The background parameters (1-3) were essentially similar for Cohort IV and the full Phase I study. The data is limited, but the measured endpoints (4-6) show a clear trend towards improved outcome in Cohort IV compared to the full study and is also encouraging when seen in relation to the most appropriate published comparator data.

# BILE DUCT CANCER

## ► About comparator data for inoperable bile duct cancer

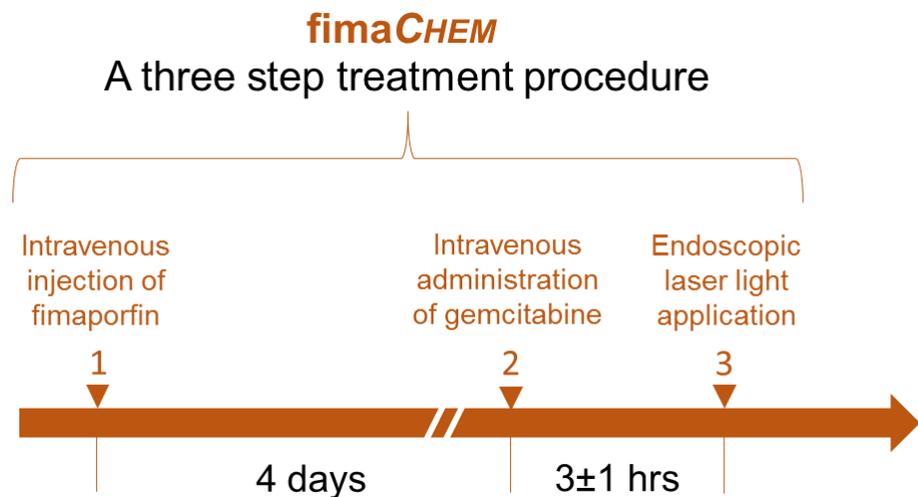
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The median overall survival (mOS) in the studies that established gemcitabine and cisplatin as standard treatment in cholangiocarcinoma (CCA) was 11.7 and 11.2 months respectively (Valle et al. NEJM (2010) 362:1273-81 and Okusaka et al. BJC (2010) 103:469-74). Gallbladder cancer patients had a poorer outcome in the latter study and the mOS was 13 months when these patients were excluded.

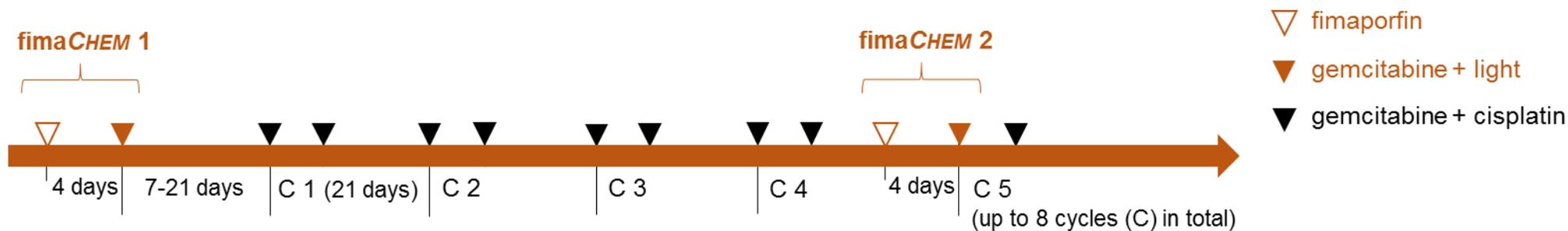
These results represent the best available published comparator data, but are not directly comparable to the data in the fimaCHEM Phase I study. The published studies include a wide range of different inoperable CCA patients, while the fimaCHEM Phase I study focuses on inoperable perihilar CCA patients.

# 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, to allow for repeated treatment in the pivotal study
  - Seven patients have been included in the study
  - Four patients have so far passed the safety window, which includes approx. 3 weeks after the second **fimaCHEM**
  - Data not yet mature enough for efficacy evaluation
- The pivotal study will commence with up to two scheduled treatments, with IDMC<sup>a</sup> performing a safety review when eight pivotal study patients have received two treatments

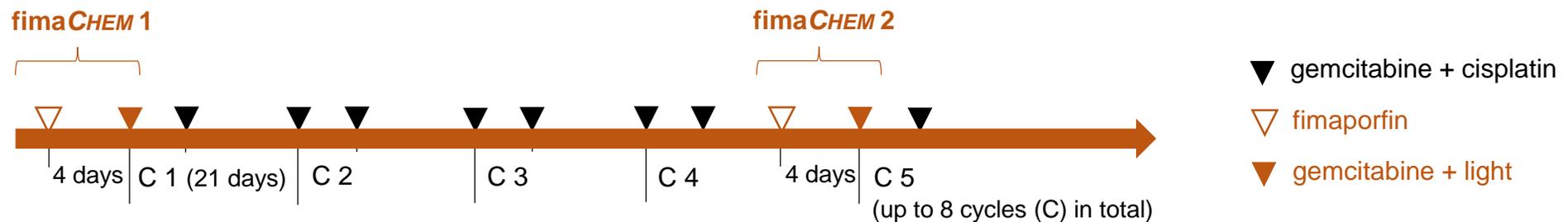


<sup>a</sup> IDMC: Independent Data Monitoring Committee

# BILE DUCT CANCER – PIVOTAL STUDY

► Randomised study with interim analysis for potential accelerated/conditional approval

- Randomised pivotal study in newly diagnosed patients with inoperable extrahepatic bile duct cancer +/- liver metastases
- Study design based on thorough discussions with the EMA<sup>a</sup> and the US FDA<sup>b</sup>
- Will involve approx. 40 key hospital sites across Europe and USA
- Approx. 36 months to interim and 50 months to final analysis
- Randomisation (1:1) of 186 patients to treatment with either fimaCHEM + SoC<sup>c</sup> or SoC only
- Primary endpoint: Progression Free Survival (PFS), with Overall Survival (OS) as key secondary
- Interim analysis primary endpoints: PFS followed by Objective Response Rate (ORR)
- IDMC<sup>d</sup> ongoing review, but no formal futility stop



<sup>a</sup> EMA: European Medicines Agency; <sup>b</sup> FDA: Food and Drug Administration; <sup>c</sup> SoC: standard of care treatment with gemcitabine + cisplatin; <sup>d</sup> IDMC: Independent Data Monitoring Committee

# 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 enhancement of antigen specific T-cell response at tolerable doses, with earlier responses and higher response rates
    - Vast number of study samples available – near-term focus on characterisation of the immune response
  - Part 3 TBD
  - Expected study completion: 2H 2018

Vaccination features: **fima VACC**

- Enhanced T-cell responses*
- High T-cell response rates*
- Early T-cell responses*



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

# RESEARCH COLLABORATIONS

## ► Six active collaborations within nucleic acid therapeutics and vaccination

		<ul style="list-style-type: none"> <li>- Collaboration initiated 2Q 2015</li> <li>- Listed on Nasdaq, developing innovative therapeutic siRNA</li> <li>- Collaboration expanded to immuno-oncology following RXi's MirlImmune acquisition</li> </ul>
	<p><b>Top-10 large pharma</b></p>	<ul style="list-style-type: none"> <li>- Collaboration initiated 3Q 2015</li> <li>- A global leader in nucleic acid therapeutics</li> <li>- Collaboration expanded to include <i>in vivo</i> studies and duration to end 2018</li> </ul>
		<ul style="list-style-type: none"> <li>- Collaboration initiated 3Q 2016</li> <li>- German biotech company developing individualised cancer immunotherapies</li> <li>- Clinical programmes in melanoma, head &amp; neck, breast, ovarian and pancreatic cancer</li> </ul>
		<ul style="list-style-type: none"> <li>- Collaboration initiated 4Q 2016</li> <li>- Belgian biotech with proprietary TriMix platform programming dendritic cells</li> <li>- Clinical programmes in melanoma and triple negative breast cancer</li> </ul>
		<ul style="list-style-type: none"> <li>- Collaboration initiated 2Q 2018</li> <li>- A listed Canadian clinical stage immunotherapy biotech</li> <li>- Multiple clinical-stage programmes in cancer and infectious diseases</li> </ul>
		<ul style="list-style-type: none"> <li>- Collaboration initiated 3Q 2018</li> <li>- A listed Danish clinical stage immunotherapy biotech</li> <li>- Multiple clinical-stage programmes in cancer and infectious diseases</li> </ul>
		<ul style="list-style-type: none"> <li>- Collaboration initiated 1Q 2016</li> <li>- Norwegian immunotherapy company</li> <li>- Therapeutic cancer vaccine against human telomerase</li> </ul>

# BILE DUCT CANCER – PIVOTAL STUDY

## ► Use of Proceeds – until filing in 2022 (4 years)

Estimated uses per programme 2019-2022 (NOK million)				
<i>fima</i> <b>CHEM</b>	<i>fima</i> <b>VACC</b>	<i>fima</i> <b>NAC</b>	General corporate purposes	Total
270-290*	22-25**	3-5	15-20	310-340***

**fima** *CHEM* – expected to cover financing need to interim read of pivotal study, including marketing application filing (conditional / accelerated approval)  
– additional funding requirement to final analysis is estimated to NOK 80-90 million

**fima** *VACC* – commercial optimisation and partnering activities

**fima** *NAC* – continued opportunistic strategy, with focus on business development activities and alliance management

\* Annual SkatteFUNN grant of NOK 9 million included

\*\* Annual BIA grant of NOK 4 million for year 2019-2020 included

\*\*\* Estimated figures are subject to several risk factors (foreign exchange rate, patient inclusion rate, number- and location of sites etc). Transaction costs not included