PCI Biotech Holding ASA - First Quarter 2014

Highlights

- **Successful completion of the first light dose cohort in the modified ENHANCE study – a Phase II study in head & neck cancer patients. The study has been amended to include a light dose escalation run-in phase to optimise the intra-tumour treatment regimen and a Proof of Concept part to confirm safety and efficacy.**

- **Started inclusion of patients in the Phase I/II Proof of Concept study with Amphinex in combination with gemcitabine in patients with inoperable bile duct cancer (cholangiocarcinoma). Evaluation of the first dose cohort is successfully completed and enrolment for the second dose cohort has been initiated.**

- **Awarded NOK 12.5 million in a BIA grant from The Research Council of Norway for the project "Development of photochemical internalization to enhance the effect of therapeutic and prophylactic vaccines".**

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Operational Review

Progress in development programs

PCI Biotech Holding ASA (PCI Biotech) is an oncology-focused company developing innovative products for cancer treatment. The products are based on PCI Biotech's patented technology, photochemical internalization (PCI). The PCI technology can enhance the effect of anticancer drugs by targeted, light-directed drug delivery into cancer cells, and can also be used as a platform that may both potentiate the effect of vaccines and enable macromolecules to reach intracellular targets.

Amphinex® in combination with bleomycin, head & neck cancer

PCI Biotech's lead candidate is the photosensitiser Amphinex. A Phase I study of Amphinex in combination with the cytotoxic agent bleomycin in cancer patients, and an extension to this study, have been completed at University College Hospital (UCH) in London. A total of 22 patients were treated in these studies, with the majority being head & neck cancer. A strong response to treatment was seen in all patients and Amphinex seemed to be well tolerated.

Phase II study in head & neck cancer patients – the ENHANCE study

The ENHANCE study is a single arm, multi-centre, phase II study to evaluate the safety and efficacy of Amphinex in combination with the generic cytotoxic agent bleomycin with superficial and interstitial laser light application. The target population is patients with recurrent head & neck squamous cell carcinoma unsuitable for surgery and radiotherapy. The study will include approximately 80 patients with progression free survival at 6 months as the primary endpoint. Patient inclusion started in May 2012.

Two different light application procedures are used in the study; surface and intra-tumour illumination. Findings from some of the patients included in the study indicated that treatment with intra-tumour illumination causes stronger local treatment effects than expected and desired and stronger treatment effects than previously observed with surface illumination in the phase I study.

The intra-tumour illumination procedure is therefore being optimized in a separate part of the study, running in parallel to the open inclusion of patients for surface illumination. The Amphinex dose has not been modified; the optimisation is performed solely by modifying the light dose. Total number of patients in the dose optimisation part of the study will depend on the number of light dose escalations needed to find an effective and safe light dose. The first patient in the light dose escalation part of the study was included in 3Q 2013 and the treatment evaluation of the first light dose cohort (3 patients) was available January 2014. No safety concerns were raised and a clear but insufficient tumour response was seen at this light dose level. A Dose Review Committee (DRC) of clinical experts and company representatives has been established to evaluate the results and provide recommendation for the continuation of the study. The DRC recommended that the light dose is escalated according to the protocol and patient inclusion at the next light dose level is currently on-going. Proof of Concept (PoC) of efficacy and safety for intra-tumour treatment and final confirmation of light dose for the ENHANCE study will be achieved by inclusion of 12 patients at the selected light dose. The company is actively working to speed up patient inclusion and a process to open further sites in selected European countries is on-going. The PoC part of the study may be completed in 2014, depending on the number of light dose escalations needed. The company will continue to update the market through press releases at the completion of light dose cohorts in the intra-tumour illumination part of the study.

A market survey performed by Bridgehead International for PCI Biotech shows a total of 110.000 – 120.000 new incidents of head and neck cancer patients in the five major markets in Europe and the United States per year. Approximately 20% of these patients are expected to be eligible for Amphinex. The promising results from the phase I study and the treatment benefits of PCI represent a significant commercial potential for the company, in light of the unmet medical need for better local treatment options and the potential market.

Clinical study in patients with inoperable bile duct cancer (Cholangiocarcinoma)

A Proof of Concept study for the use of PCI in patients with inoperable bile duct cancer is initiated. In this indication Amphinex will be used in combination with the generic cytotoxic agent gemcitabine.

The Proof of Concept study is an open-label, multi-centre phase I/II study in up to 45 patients to assess the safety and efficacy of Amphinex induced PCI of gemcitabine, followed by systemic cisplatin/gemcitabine in patients with inoperable bile duct cancer. The study consists of a dose escalation/phase I part to assess tolerance of local bile duct treatment with Amphinex induced PCI of gemcitabine and a randomized double-arm phase II part. In phase II patients will be randomized to either a control arm (stenting alone followed by gemcitabine/cisplatin chemotherapy) or the PCI arm (stenting followed by Amphinex induced PCI treatment of gemcitabine followed by gemcitabine/cisplatin chemotherapy). The randomisation ratio for this study is 2.5:1 in favor of the PCI arm. The phase I primary objective is to determine a tolerable dose for local bile duct treatment with Amphinex induced PCI of gemcitabine, while the phase II primary objective is to assess efficacy in terms of progression free survival. The first patient was included in January 2014 at Aintree University Hospital in Liverpool, UK, and the treatment evaluation of the first dose cohort (3 patients) was completed in April 2014. No safety concerns were observed at this dose level. As the phase I primary objective is to determine a tolerable dose, no efficacy results are available at this stage. The company is actively working to speed up patient inclusion and a process to open further sites in selected European countries is on-going. The phase I part of the study may be completed in 2014, depending on the number of dose escalations needed. The company will continue to update the market through press releases at the completion of dose cohorts.

Available market information indicates approximately 11,000 new incidents of patients in the United States and the largest markets in Europe per year and about 20% of these patients are expected to be eligible for PCI treatment. The unmet medical need for better local treatment options and the fact that bile duct cancer is a rare disease that can achieve specific marketing benefits as an orphan indication, represent a significant commercial potential for the company, in view of PCI treatment benefits.

PCI for vaccination

The company has increased the activity level to document and optimise the PCI effect for therapeutic vaccines, i. e. vaccines that aim to treat an already established disease in the patient. This work involves cooperations with NTNU in Trondheim, Norway, The Norwegian Radium Hospital, Oslo, Norway and University Hospital Zürich, Switzerland. The company has in support and expansion of this work been awarded NOK 12.5 million in a BIA grant from The Research Council of Norway for the project "Development of photochemical internalization to enhance the effect of therapeutic and prophylactic vaccines". The project goal is to document that the PCI technology can be used to improve the efficacy of vaccines. The main focus of the project will be to verify and further develop the technology for use in therapeutic vaccines against cancer, but the project also includes use of the PCI technology by vaccination against certain types of virus and bacterial infections.

The two most important components in the immunological reaction to vaccines are the antibody and the cellular cytotoxic responses. For many vaccines, and especially for therapeutic vaccines, a strong cellular response is of great importance. A possible benefit when applying PCI within vaccination is that PCI can direct the immunological response towards a stronger cellular response. This could be important for the effect of therapeutic vaccines for example within cancer.

Proof-of-principle has been established in a mouse model for enhancement of *ex vivo* vaccination. *Ex vivo* (also called autologous) vaccination is a treatment procedure where immune cells are removed from the patient and treated outside the body, where PCI can be included in the treatment. The treated immune cells are then reintroduced to the patient. Proof-of-principle has also been established in mouse models for enhancement of *in vivo* vaccination. These promising preclinical results have been achieved by simply mixing the antigen and photosensitiser for local injection, and then illuminating locally with an inexpensive light source. The preclinical proof-of-principle results have in 2013 been published in renowned scientific journals

Effective adjuvant technologies are considered key to the success of therapeutic vaccination, and vaccination companies are seeking improved adjuvant technologies for their vaccine technologies. PCI Biotech's novel mode of action may allow the use of PCI as a new adjuvant system for vaccinations where existing adjuvant technologies do not work. There are a large number of therapeutic cancer vaccines under development and available market information shows an expected global market of more than 7 billion U.S. dollars in 2019. The prophylactic vaccines market is more mature and consist of few players, but also here PCI may play a central role for companies seeking new solutions. The company has initiated discussions with potential partners who have shown interest in PCI for vaccines.

PCI for macromolecules

PCI has the potential to increase the effect of different types of macromolecules, e.g. siRNA and Anti-body Drug Conjugates (ADC). As part of the increased focus on partnering activities, the company has initiated discussions with potential partners that show interest in PCI for delivery of macromolecules.

Financial Review

Results 1th Quarter 2014

The company received grants from Norway and these are shown as other income. Total revenues in the quarter were NOK 2.0 million compared with NOK 1.4 million in Q1 2013.

R&D costs in Q1 2014 were NOK 10.0 million compared with NOK 8.0 million in Q1 2013.

G&A costs in Q1 2014 were NOK 1.4 million compared with NOK 0.7 million in Q1 2013.

Total operating costs were NOK 11.4 million in Q1 2014, compared with NOK 8.6 million in Q1 2013.

Operating results were NOK -9.4 million in Q1 2014 compared with NOK -7.2 million in Q1 2013.

Cash flow from operations was NOK -8.3 million in Q1 2014, compared with NOK -7.1 million in Q1 2013. Net cash flow was NOK -8.2 million in Q1 2014, compared with NOK -6.7 million in Q1 2013.

Balance

The company held cash and cash equivalents of NOK 38.4 million at the end of the quarter. Total equity was NOK 34.7 million compared with NOK 43.4 million at the end of 2013. The change in equity reflects the loss in the period and a NOK 0.6 million positive equity effect from the share option scheme.

Strategy

PCI Biotech's strategy within the various business areas is to prioritize commercialization through agreements with external partners. The company's goal is to establish partnerships based on results from the phase II part of the ongoing clinical studies, and the potential phase III part will be performed in cooperation with or by other players within the field of oncology. The possibility of entering into partnerships depends on the quality of phase II results. Within vaccines and macromolecules PCI Biotech's strategy is to use preclinical results to enter into various agreements for further development and use of PCI as a platform technology.

Outlook

PCI Biotech will continue to focus on the clinical development of Amphinex in combination with cancer drugs for localised cancer treatment, based on the company's unique PCI technology. The company will increase the activity level in pre-clinical development and licensing of PCI as a versatile and innovative platform for enhancement of vaccines and delivery of macromolecules.

The main priorities with the available funds are to:

- Effectively progress the light dose optimization and proof of concept of intra-tumour head and neck cancer treatment of Amphinex and bleomycin;
- Complete the first part of the proof of concept study of bile duct cancer treatment with Amphinex and gemcitabine;
- Solidify a robust vaccination IP estate and further strengthen the promising preclinical results;
- Partnering activities across all commercially interesting areas for the PCI platform.

CONDENSED INTERIM CONSOLIDATED FINANCIAL INFORMATION

PROFIT AND LOSS

(In NOK 1,000)	Note	Q1 2014	Q1 2013	01.01- 31.12 2013
Other Income	5	**1 967**	**1 391**	**6 681**
Research and development	8	9 959	7 972	32 789
General and administrative		1 417	663	3 217
Operating costs		**11 376**	**8 635**	**36 006**
Operating results		**-9 409**	**-7 244**	**-29 325**
Financial income and costs				
Financial income		283	419	1 717
Financial expenses		-122	0	0
Net financial result		**161**	**419**	**1 717**
Ordinary profit before taxes		**-9 248**	**-6 825**	**-27 608**
Tax on ordinary result	9	0	0	0
Net profit/loss	4	**-9 248**	**-6 825**	**-27 608**
Other comprehensive income		0	0	0
Comprehensive income		**-9 248**	**-6 825**	**-27 608**

BALANCE SHEET

(In NOK 1,000)	Note	31.03 2014	31.03 2013	31.12 2013
Fixed and intangible assets				
Operating assets		17	22	18
Total fixed and intangible assets		**17**	**22**	**18**
Current assets				
Short term receivables	*7*	4 586	5 057	6 123
Cash & cash equivalents	*7*	38 433	66 343	46 595
Total current assets		**43 020**	**71 400**	**52 718**
Total assets		**43 037**	**71 422**	**52 736**
Shareholders equity and liabilities				
Shareholders equity				
Paid in capital		99 911	190 808	99 911
Other reserves		-65 195	-127 486	-56 515
Total equity	*10*	**34 716**	**63 322**	**43 396**
Trade debtors		1 648	2 600	4 061
Other short term debt		6 673	5 500	5 279
Total debt		**8 321**	**8 100**	**9 340**
Total shareholders equity and liabilities		**43 037**	**71 422**	**52 736**

CHANGE IN SHAREHOLDERS EQUITY

(In NOK '000)	Note	Paid in capital	Share premium	Other paid in capital	Retained earnings	Total
Balance at 31 December 2012		**22 999**	**76 524**	**94 306**	**-124 122**	**69 706**
Capital increase		180	208	-	-	388
Share option scheme	10	-	-	909	-	909
Comprehensive income in the period		-	-	-	-27 608	-27 608
Allocation		-	-	-95 215	95 215	-
Balance at 31 December 2013		**23 179**	**76 732**	**-**	**-56 515**	**43 396**
Share option scheme	10	-	-	568	-	568
Comprehensive income in the period		-	-	-	-9 248	-9 248
Allocation		-	-	-568	568	-
Balance at 31 March 2014		**23 179**	**76 732**	**-**	**-65 195**	**34 716**

CASH FLOW

(In NOK '000)	Note	Q1 2014	Q1 2013	01.01-31.12 2013
Ordinary profit before taxes		-9 248	-6 825	-27 608
Depreciation, Amortization and Write Off		1	-	4
Share options		568	440	909
Net financials		-161	-419	-1 717
Changes in working capital		517	-333	-181
Cash flow from operations		-8 323	-7 137	-28 593
Net financials		161	419	1 717
Taxes paid		-	-	-
Net cash flow from operations		-8 162	-6 718	-26 876
Cash flow from investments				
Purchase of tangible assets		-	-22	-
Net cash flow from investments		-	-22	-
Cash flow from financial activities				
Net proceeds from share issues		-	-	388
Net cash flow from financial activities		-	-	388
Net change in cash during the period		-8 162	-6 740	-26 488
Cash and cash equivalents at the beginning of the period		46 595	73 083	73 083
Cash and cash equivalents at the end of the period		38 433	66 343	46 595

SELECTED EXPLANATORY NOTES:

1. Nature of operation

PCI Biotech Holding ASA (PCI Biotech) was established in 2008, and comprises PCI Biotech Holding ASA, the fully owned subsidiary PCI Biotech AS and the Islandic Branch PCI Biotech Utibu. PCI Biotech AS was a subsidiary of Photocure ASA until June 2008. The PCI Biotech shares have been listed on the Oslo Axess since 18 June 2008 under the ticker PCIB. The company is headquartered at Lysaker, Norway.

PCI Biotech has developed a unique and patented photochemical intracellular drug delivery technology for use in cancer therapy and other diseases. The technology may also be used to enhance the immunological response of vaccines. The company collaborates closely with The Norwegian Radium Hospital in Oslo, Norway and receives substantial funding on several projects from the Research Council of Norway. The company has an extensive international collaboration network with recognised expert groups in both drug delivery and vaccination. PhotoChemical Internalisation (PCI) is a proprietary technology for light-directed intracellular drug delivery by triggered endosomal release.

The PCI technology has potential to improve the effect both of existing drugs and new classes of drugs, such as therapeutic vaccines, gene therapy and other therapies based on nanotechnology or on biotechnological principles. The company's objective is to prove the clinical usefulness of the technology with different drugs and subsequently license out the technology to partners for further development and marketing. Revenues will be generated at the time of partnering and onwards from up-front payments, milestone payments and royalties from licensees. PCI Biotech focuses on the development of PCI products for enhanced delivery of marketed cancer drugs, and as a platform that may both potentiate the effect of vaccines and enable macromolecules to reach intracellular targets. PCI Biotech has two active clinical studies with the lead candidate Amphinex: a Phase II trial in head & neck cancer with the cytotoxic agent bleomycin and a Phase I/II trial in bile duct cancer with the cytotoxic agent gemcitabine. The company has an on-going preclinical program to document the use of PCI to enhance and direct the immune response of vaccines towards a stronger cellular response.

2. Basis of presentation

These Interim Financial Statements should be read in conjunction with the Consolidated Financial Statements for the year ended 31 December 2013 (hereafter 'the Annual Financial Statements'), as they provide an update of previously reported information. They were approved for issue by the Board of Directors on 24 March 2014. The accounting policies used are consistent with those used in the Annual Financial Statements. The presentation of the Interim Financial Statements is consistent with the Annual Financial Statements. The interim report has not been subject to an audit. The board of directors approved the interim condensed financial information on 12 May 2014.

3. Summary of significant accounting policies

The accounting policies applied and the presentation of the interim condensed consolidated financial information is consistent with the consolidated financial statements for the year ended 31 December 2013.

The new standards, interpretations or amendments to published standards that were effective for the annual period beginning on January 1, 2014 and that could affect the PCI Biotech are discussed in accounting policies, part 3, to the consolidated financial statements for 2013. In the 2013 financial statements, PCI Biotech made evaluations that none of these are expected to have significant effect for PCI Biotech.

4. Earnings per share

Earnings per share:

	Q1 2014	Q1 2013	FY 2013
Result allocated to shareholders (in NOK '000)	(9 248)	(6 825)	(27 608)
Weighted average of outstanding shares (in '000)	7 726	7 666	7 696
Earnings per share (NOK per share)	-1,20	-0,89	-3,59

Diluted earnings per share:

	Q1 2014	Q1 2013	FY 2013
Result allocated to shareholders (in NOK '000)	(9 248)	(6 825)	(27 608)
Weighted average of outstanding shares (in '000)	8 195	8 155	7 696
Earnings per share (NOK per share)	-1,20	-0,89	-3,59

Weighted average of outstanding diluted shares is weighted number of average shares adjusted with share options. Earning per share is not affected by the dilution if negative results in the period.

5. Segment information

The Company reports only one segment and revenues are not influenced by any cyclicality of operations. The company received grants from Norway and EU and these are shown as other income.

6. Related party transactions

PCI Biotech is relying on services provided by third parties, included related parties, as a result of its organisational set-up. PCI Biotech considers that its business relationship with The Norwegian Radium Hospital Research Foundation and legal services provided by Board member Theresa Comiskey Olsen represents related party transactions. The following table shows the extent of such transactions in the reported periods (all figures in NOK '000):

Purchase of services	Q1 2014	Q1 2013	FY 2013
The Norwegian Radium Hospital Research Foundation	586	445	1 600
Theresa Comiskey Olsen	30	0	33

At the end of the quarter, PCI Biotech had NOK 0.2 million in short term debt to The Norwegian Radium Hospital Research Foundation and no short term debt to Theresa Comiskey Olsen.

7. Credit risk, foreign currency risk and interest risk

Credit risk

PCI Biotech trades only with recognised, creditworthy third parties, of which most are governmental institutions. Receivable balances are monitored on an on going basis with the result that the company's exposure to bad debts is not significant and therefore no offset of bad debts has been recognised at the end of the quarter.

Maturity profile on receivables as per end of the quarter:

	Not due	Less than 3 months	3 to 12 months	Total
Trade receivables	-	-	-	-
Other receivables	4 586	-	-	4 586
Total receivables	**4 586**	**-**	**0**	**4 586**

A majority of other receivables relates to accrued, not received grants.

Foreign currency risk
PCI Biotech has transactional currency exposure arising from purchases in currencies other than the functional currency (NOK). PCI Biotech has not implemented any hedging strategy to reduce currency risk.

Interest risk
PCI Biotech has no interest bearing debt. At end of the quarter, NOK 20 million of the cash was placed at accounts with fixed interest. The fixed interest matures in Q2 2014.

8. Research and Development costs

	Q1 2014	Q1 2013	FY 2013
Clinical studies	3 724	4 436	16 724
Pre-clinical studies	2 880	1 526	6 742
CMC and equipment	2 050	1 723	7 391
Patents	1 305	287	1 931
Other costs	0	0	0
Total	**9 959**	**7 972**	**32 789**

9. Deferred tax and deferred tax assets

At the end of the quarter, the company held NOK 49.9 million in non-capitalised deferred tax assets.

10. Share options

No changes are made to the employee option scheme program in Q1 2014. Remaining share options outstanding at the end of the period have the following expiry date and exercise prices:

	Exercise price in NOK per	Number of shares	
Expiry date	share	31.03.2014	31.12.2013
2014 - Q3	6.47	174 000	174 000
2015 - Q3	37.24	90 000	90 000
2016 - Q3	19.02	170 000	170 000
2017 - Q3	37.02	86 500	86 500
2018 - Q3	19.63	85 000	85 000
2018 - Q3	18.64	40 000	40 000
Total		**645 500**	**645 500**

11. Material events subsequent to the end of the reporting period

To the best of PCI Biotech's knowledge, there have been no events subsequent to the end of the reported interim period that would influence the interim financial statement.