PCI Biotech Holding ASA - Fourth Quarter 2013 and preliminary full year 2013 Report

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 bile duct cancer (cholangiocarcinoma).**

- **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".**

- **Strengthened the PCI vaccination patent estate by the filing of several patent applications within PCI-mediated immunisation, supplementing already granted and pending patents in this area.**

- **Achieved preclinical Proof of Principle for the use of PCI-mediated immunisation both *in vivo* and *ex vivo*. Studies have been published in renowned scientific journals.**

- **Strengthened the business development function and increased focus on partnering activities across several commercially interesting areas for utilisation of the company's patented PCI-technology.**

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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 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.

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 superficial 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.

Clinical study in patients with bile duct cancer (Cholangiocarcinoma)

A Proof of Concept study for the use of PCI in patients with bile duct cancer has been 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 the tolerance of local bile duct treatment 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. 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.

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 cooperation 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 to 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. This principle is employed in the only cancer vaccine that is approved for use in humans, and it is also the basis for several cancer vaccines that are under clinical development. Proof-of-principle has also been established in a mouse model for enhancement of *in vivo* vaccination, where up to 40 times PCI induced enhancement of antigen specific T-cells has been seen. 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; the *ex vivo* results in European Journal of Pharmaceutics and Biopharmaceutics, and the *in vivo* results in Journal of Controlled Release.

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. During 2013, several patent applications were filed within the vaccination area. These applications will give PCI Biotech the opportunity to obtain further patent protection for the use of the PCI technology in vaccination, supplementing the already granted and pending patents within this area.

PCI represents a simple and innovative adjuvant platform that may be licensed in an innovative emerging cancer vaccine market in need of novel solutions. The company has initiated discussions with potential partners that show interest in PCI in relation to 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.

Organisational changes

The business development function has been strengthened by the appointment of Gaël L'Hévéder (44) as Chief Business Development Officer. Mr L'Hévéder was hired 2Q 2013 and has more than 15 years industry experience gained in both large pharmaceutical companies such as Sanofi-Aventis, Baxter and Roche, and in biotech start-ups. He is a dual French/US citizen and he holds a Master of Science in organic chemistry from Université Louis Pasteur, Strasbourg, France.
Ronny Skuggedal (38) has been appointed new CFO in PCI Biotech Holding ASA in Q3 2013. Mr Skuggedal comes from the position as director in PwC where he has been an auditor and advisor since 2001. He holds a Master of Business and Economics from Norwegian School of Economics (NHH) and Master of Auditing and Accounting from Norwegian Business School (BI). Ronny is a State Authorized Public Accountant in Norway.

Financial Review

Results 4th Quarter 2013

The company received grants from Norway and EU and these are shown as other income. Total revenues in the fourth quarter were NOK 2.0 million compared with NOK 1.4 million in Q4 2012.

R&D costs in Q4 2013 were NOK 10.1 million compared with NOK 8.6 million in Q4 2012.

G&A costs in Q4 2013 were NOK 1.2 million compared with NOK 1.6 million in Q4 2012.

Total operating costs were NOK 11.3 million in Q4 2013, compared with NOK 10.2 million in Q4 2013.

Operating results were NOK -9.3 million in Q4 2013 compared with NOK -8.8 million in Q4 2012.

Cash flow from operations was NOK -6.4 million in Q4 2013, compared with NOK -5.3 million in Q4 2012. Net cash flow was NOK -5.9 million in Q4 2013, compared with NOK -4.9 million in Q4 2012.

Preliminary Results 2013

The company received grants from Norway and EU and these are shown as revenues. Revenues were NOK 6.7 million in 2013 compared with NOK 6.8 million in 2012. Total operating costs were NOK 36.0 million in 2013, compared with NOK 34.1 million in 2012.

R&D costs in 2013 were NOK 32.8 million, compared with NOK 31.3 million in 2012. G&A costs in 2013 were NOK 3.2 million compared with NOK 2.9 million in 2012.

Operating results were NOK -29.3 million in 2013 compared with NOK -27.4 million in 2012.

Cash flow from operations was NOK -28.6 million in 2013, compared with NOK -24.4 in 2012. Net cash flow was NOK -26.9 million in 2013, compared with NOK -22.0 million in 2012.

Balance

The company held cash and cash equivalents of NOK 46.6 million at the end of the quarter. Total equity was NOK 43.4 million compared with NOK 69.7 million at the end of 2012. The change in equity reflects the loss in the period and a NOK 0.4 million capital increase through exercising of share options in Q3 2013.

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	Q4 2013	Q4 2012	01.01- 31.12 2013	01.01-31.12 2012
Other Income	5	**1 986**	**1 398**	**6 681**	**6 765**
Research and development	8	10 102	8 565	32 789	31 263
General and administrative		1 207	1 634	3 217	2 856
Operating costs		**11 309**	**10 199**	**36 006**	**34 119**
Operating results		**-9 323**	**-8 801**	**-29 325**	**-27 354**
Financial income and costs					
Financial income		474	505	1 717	2 322
Financial costs		0	67	0	227
Net financial result		**474**	**438**	**1 717**	**2 095**
Ordinary profit before taxes		**-8 849**	**-8 363**	**-27 608**	**-25 259**
Tax on ordinary result	9	0	0	0	0
Net profit/loss	4	**-8 849**	**-8 363**	**-27 608**	**-25 259**
Other comprehensive income		0	0	0	0
Comprehensive income		**-8 849**	**-8 363**	**-27 608**	**-25 259**

BALANCE SHEET

(In NOK 1,000)	Note	31.12 2013	31.12 2012
Non-current assets			
Operating assets		18	0
Total non-current assets		**18**	**0**
Current assets			
Short term receivables	*7*	6 123	5 118
Cash & cash equivalents		46 595	73 083
Total current assets		**52 718**	**78 201**
Total assets		**52 736**	**78 201**
Shareholders equity and liabilities			
Shareholders equity			
Paid in capital		99 911	191 579
Other reserves		-56 515	-121 873
Total equity	*10*	**43 396**	**69 706**
Trade debtors	*6*	4 061	1 984
Other short term debt		5 279	6 511
Total debt		**9 340**	**8 495**
Total shareholders equity and liabilities		**52 736**	**78 201**

CHANGE IN SHAREHOLDERS EQUITY

(In NOK '000)	Note	Paid in capital	Share premium reserve	Other paid in capital	Retained earnings	Total
Balance at 31 December 2011		**22 999**	**76 524**	**91 875**	**-98 863**	**92 533**
Share option scheme	10	-	-	2 431	-	2 431
Comprehensive income in the period		-	-	-	-25 259	-25 259
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**

CASH FLOW

(In NOK '000)	Note	Q4 2013	Q4 2012	01.01-31.12 2013	01.01-31.12 2012
Ordinary profit before taxes		-8 849	-8 363	-27 608	-25 259
Depreciation, Amortization and Write Off		1	-	4	17
Share options		429	1 211	909	2 431
Net financials		-474	-438	-1 717	-2 322
Changes in working capital		2 487	2 260	-181	779
Cash flow from operations		-6 406	-5 330	-28 593	-24 354
Net financials		474	438	1 717	2 322
Taxes paid		-	-	-	-
Net cash flow from operations		-5 932	-4 892	-26 876	-22 032
Cash flow from investments					
Purchase of tangible assets		-	-	-	-
Purchase of intangible assets		-	-	-	-
Net cash flow from investments		-	-	-	-
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		-5 932	-4 892	-26 488	-22 032
Cash and cash equivalents at the beginning of the period		52 527	77 975	73 083	95 115
Cash and cash equivalents at the end of the period		46 595	73 083	46 595	73 083

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 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 Norwegian Research Council. 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.

The PCI Biotech shares have been listed on the Oslo Axess since 18 June 2008 under the ticker PCIB.

2. Basis of presentation

These Interim Financial Statements should be read in conjunction with the Consolidated Financial Statements for the year ended 31 December 2012 (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 11 March 2013. 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 24 February 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 2012.

The new standards, interpretations or amendments to published standards that were effective for the annual period beginning on January 1, 2013 and that could affect the PCI Biotech are discussed in accounting policies, part 3, to the consolidated financial statements for 2012. In the 2012 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:

	Q4 2013	Q4 2012	FY 2013	FY 2012
Result allocated to shareholders (in NOK '000)	(8 849)	(8 363)	(27 608)	(25 259)
Weighted average of outstanding shares (in '000)	7 726	7 666	7 696	7 666
Earnings per share (NOK per share)	-1,15	-1,09	-3,59	-3,29

Diluted earnings per share:

	Q4 2013	Q4 2012	FY 2013	FY 2012
Result allocated to shareholders (in NOK '000)	(8 849)	(8 363)	(27 608)	(25 259)
Weighted average of outstanding shares (in '000)	8 137	8 155	8 165	8 155
Earnings per share (NOK per share)	-1,15	-1,09	-3,59	-3,29

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 Radiumhospitalets Forskningsstiftelse 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	Q4 2013	Q4 2012	FY 2013	FY 2012
Radiumhospitalets Forskningsstiftelse	428	424	1 582	1 593
Theresa Comiskey Olsen	17	0	20	3

At the end of the quarter, PCI Biotech had NOK 0.1 million in short term receivables on Radiumhospitalets Forskningsstiftelse 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 31 December:

	Not due	Less than 3 months	3 to 12 months	Total
Trade receivables	3	0	0	3
Other receivables	6 120	0	0	6 120
Total receivables	**6 123**	**0**	**0**	**6 123**

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 Q1 2014.

8. Research and Development costs

	Q4 2013	Q4 2012	FY 2013	FY2012
Clinical studies	4 212	4 408	16 724	15 938
Pre-clinical studies	2 144	780	6 742	5 308
CMC and equipment	3 241	1 807	7 391	5 840
Patents	504	435	1 931	3 041
Other costs	0	1 135	0	1 135
Total	**10 102**	**8 565**	**32 789**	**31 263**

9. Deferred tax and deferred tax assets

At the end of the quarter, the company held NOK 46.9 million in non-capitalised deferred tax assets.

10. Share options

In 2013, the following changes are made to the option program;

• Options allocated in 2008 expiring in 2013 were extended with 3 years until 2016. At the same time, options allocated in 2008 were reduced with 1/3 from 255,000 to 170,000 options. Strike price is unchanged at NOK 19.02 per share. The fair value of this change using the Black-Scholes valuation model was NOK 725,000. The significant input to the model were a share price of NOK 19,63 at the grant day, volatility of 83% and risk free rate of 1.54 % for the prolonged period and volatility of 55% and risk free rate of 1.44 % for the released options. Dividend yield 0% in both calculations.

• The 85,000 released options were allocated to 2 employees. The employees may exercise 1/3 of the options after 1 year, another 1/3 after 2 years and the last 1/3 after 3 years. The options expire in Q3 2018. Strike price for these share options is NOK 19.63 per share, equal to the average price of all trades the 5 last days with trade prior to allocation. The fair value of this allocation using the Black-Scholes valuation model was NOK 888,000, The significant input to the model were a share price of NOK 19,63 at the grant day, volatility of 83%, dividend yield 0%, expected duration of 3 years and risk free rate of 1.54 %.

In Q3 2013 two employees resigned and a total number of 68 500 share options were canceled. Share option cost, related to these canceled share options, charged in previous periods are accounted for as change of estimates according to *IFRS 2 Share-based payments* resulting in a positive P&L effect of approximately NOK 1 million in Q3 2013.

In Q4 2013 40,000 options was allocated to the current CFO Ronny Skuggedal. The employee may exercise 1/3 of the options after 1 year, another 1/3 after 2 years and the last 1/3 after 3 years. The options expire in Q3 2018. Strike price for these share options is NOK 18.64 per share, equal to the average price of all trades the 5 last days with trade prior to allocation. The fair value of this allocation using the Black-Scholes valuation model was NOK 400,000, The significant input to the model were a share price of NOK 18,64 at the grant day, volatility of 83%, dividend yield 0%, expected duration of 3 years and risk free rate of 1.90 %.

On 28th June, the CFO at that time, Bernt Olav Røttingsnes exercised 60,000 options allocated in Q2 2009 with an exercise price of NOK 6.47 per share. The capital increase was completed in July 2013.

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**	**31.12.2013**	**31.12.2012**
2013 - Q3	19.02	0	255 000
2014 - Q3	6.47	174 000	234 000
2015 - Q3	37.24	95 000	115 000
2016 - Q3	19.02	170 000	0
2017 - Q3	37.02	86 500	135 000
2018 - Q3	19.63	85 000	0
2018 - Q3	18.64	40 000	0
Total		**650 500**	**739 000**

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 financial statements included in this report.