

# PCI Biotech Holding ASA - Third Quarter 2013 Report

## Highlights

- **First patient included in the intra-tumour treatment run-in phase of the re-started 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.**
- **Patient inclusion into the Proof of Concept study in bile duct cancer (cholangiocarcinoma) has been difficult.. The study is being expanded to further hospitals in selected European countries.**
- **Preclinical results providing Proof of Principle for the use of PCI-mediated immunization published in the renowned “*European Journal of Pharmaceutics and Biopharmaceutics*”.**
- **Filed several patent applications for use of the PCI-technology within vaccination, supplementing the already granted and pending patents within this area.**
- **Enhanced partnering activities across several commercially interesting areas for utilisation of the 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<sup>®</sup> in combination with bleomycin, head & neck cancer**

PCI Biotech's lead candidate is the photosensitiser Amphinex. A Phase I/II 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 in 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. Preliminary findings from some of the patients included in the study indicate that treatment with intra-tumour illumination causes stronger local treatment effects than expected and desired, and stronger treatment effects than what was observed with surface illumination in the Phase I/II study at University College Hospital in London.

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 optimization 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 dose escalations needed to find an efficient and safe light dose. 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. Four European sites are currently open for inclusion and actively screening for patients for the intra-tumour part of the study.

Patient inclusion in the ENHANCE study has been slower than initially expected, due both to too strict inclusion criteria and the too strong effect with intra-tumour illumination. The company is actively working to speed up patient inclusion and a process to open further sites in selected European countries is ongoing. The PoC part of the study may be completed in 1H 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. Patient inclusion has been more difficult than expected. The company is actively working to speed up patient inclusion and a process to open further sites in selected European countries is ongoing.

#### **PCI for vaccination**

The company has increased the focus on a project that aims 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 project involves cooperation with NTNU in Trondheim, Norway, The Norwegian Radium Hospital, Oslo, Norway and University Hospital Zürich, Switzerland.

The two most important components in the immunological response to vaccines are the antibody response and the cellular response. 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 illuminate locally with an inexpensive light source.

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.

PCI represents a simple and innovative adjuvant platform that may be licensed on a non-exclusive basis in an innovative emerging cancer vaccine market in need of novel solutions. During Q3, 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. The company has initiated discussions with potential partners that show interest in evaluating 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), and as part of the increased focus on partnering activities, the company has initiated discussions with potential partners that show interest in evaluating PCI for delivery of their macromolecules.

#### **Ronny Skuggedal (37) has been appointed new CFO in PCI Biotech Holding ASA in Q3 2013.**

Ronny 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 3<sup>rd</sup> Quarter 2013**

The company received grants from Norway and these are shown as revenues. Revenues in the third quarter were NOK 1.6 million in line with NOK 1.6 million in Q3 2012.

R&D costs in Q3 2013 were NOK 8.2 million compared with NOK 9.2 million in Q3 2012.

G&A costs in Q3 2013 were NOK 0.4 million in line with NOK 0.4 million in Q3 2012.

Total operating costs were NOK 8.5 million in Q3 2013, compared with NOK 9.6 million in Q3 2012. The reduction is due to a positive P&L effect of NOK 1.0 million from cancellation of share options in Q3 2013.

Operating results were NOK -6.9 million in Q3 2013 compared with NOK -8.1 million in Q3 2012.

Net cash flow from operations was NOK -7.5 million in Q3 2013, compared with NOK -7.9 million in Q3 2012. Net cash flow in the quarter was NOK -7.1 million in Q3 2013, compared with NOK -7.9 million in Q3 2012.

### Results YTD 2013

Revenues were NOK 4.7 million YTD 2013 compared with NOK 5.4 million YTD 2012. Total costs were NOK 24.7 million YTD 2013, compared with NOK 23.9 million YTD 2012.

R&D costs YTD 2013 were NOK 22.7 million, in line with NOK 22.7 million YTD 2012. G&A costs YTD 2013 were NOK 2.0 million compared with NOK 1.2 million YTD 2012.

Operating results were NOK -20.0 million YTD 2013 compared with NOK -18.6 million YTD 2012.

Net cash flow from operations was NOK -20.9 million YTD 2013, compared with NOK -17.1 YTD 2012. Net cash flow was NOK -20.6 million YTD 2013, compared with NOK -17.1 million YTD 2012.

### Balance

The company held cash and cash equivalents of NOK 52.5 million at the end of the quarter. Total equity was NOK 51.8 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. An increased focus will also be placed on the development and licensing of PCI as a versatile and innovative adjuvant platform for vaccination.

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;
- Build a robust vaccination IP estate and further strengthen the promising preclinical results;
- Ramp up partnering activities.

## CONDENSED CONSOLIDATED FINANCIAL INFORMATION

### PROFIT AND LOSS

<i>(In NOK 1,000)</i>	Note	Q3 2013	Q3 2012	01.01- 30.09 2013	01.01-30.09 2012	01.01-31.12 2012
<b>Other Income</b>		<b>1 618</b>	<b>1 562</b>	<b>4 696</b>	<b>5 367</b>	<b>6 765</b>
Research and development	8	8 164	9 200	22 687	22 697	31 263
General and administrative		361	429	1 966	1 222	2 856
<b>Operating costs</b>		<b>8 525</b>	<b>9 629</b>	<b>24 653</b>	<b>23 919</b>	<b>34 119</b>
<b>Operating results</b>		<b>-6 908</b>	<b>-8 067</b>	<b>-19 958</b>	<b>-18 552</b>	<b>-27 354</b>
<b>Financial income and costs</b>						
Financial income		346	444	1 243	1 657	2 322
Financial costs		0	0	0	0	-227
<b>Net financial result</b>		<b>346</b>	<b>444</b>	<b>1 243</b>	<b>1 657</b>	<b>2 095</b>
<b>Ordinary profit before taxes</b>		<b>-6 562</b>	<b>-7 623</b>	<b>-18 715</b>	<b>-16 895</b>	<b>-25 259</b>
Tax on ordinary result	9	0	0	0	0	0
<b>Net profit/loss</b>	4	<b>-6 562</b>	<b>-7 623</b>	<b>-18 715</b>	<b>-16 895</b>	<b>-25 259</b>
Other comprehensive income		0	0	0	0	0
<b>Comprehensive income</b>		<b>-6 562</b>	<b>-7 623</b>	<b>-18 715</b>	<b>-16 895</b>	<b>-25 259</b>

### BALANCE SHEET

<i>(In NOK 1,000)</i>	Note	30.09 2013	30.09 2012	31.12 2012
<b>Fixed and intangible assets</b>				
Operating assets		20	0	0
<b>Total fixed and intangible assets</b>		<b>20</b>	<b>0</b>	<b>0</b>
<b>Current assets</b>				
Short term receivables	7	6 290	6 143	5 118
Cash & cash equivalents		52 527	77 975	73 083
<b>Total current assets</b>		<b>58 817</b>	<b>84 118</b>	<b>78 201</b>
<b>Total assets</b>		<b>58 837</b>	<b>84 118</b>	<b>78 201</b>
<b>Shareholders equity and liabilities</b>				
<b>Shareholders equity</b>				
Paid in capital		194 695	190 368	191 148
Other reserves		-142 836	-113 509	-96 615
<b>Total equity</b>	10	<b>51 859</b>	<b>76 859</b>	<b>69 706</b>
Trade debtors		817	2 313	1 984
Other short term debt		6 161	4 946	6 511
<b>Total debt</b>		<b>6 978</b>	<b>7 259</b>	<b>8 495</b>
<b>Total shareholders equity and liabilities</b>		<b>58 837</b>	<b>84 118</b>	<b>78 201</b>

## CHANGE IN SHAREHOLDERS EQUITY

<i>(In NOK '000)</i>	Note	Paid in capital	Share premium reserve	Other paid in capital	Retained earnings	Total
Balance at 31 December 2010		22 999	76 524	91 014	-85 114	105 423
Share option scheme	10	-	-	861	-	861
Comprehensive income in the period		-	-	-	-13 749	-13 749
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	-	-	480	-	480
Comprehensive income in the period		-	-	-	-18 716	-18 716
Balance at 30 September 2013		23 179	76 732	94 786	-142 838	51 859

## CASH FLOW

<i>(In NOK '000)</i>	Note	Q3 2013	Q3 2012	01.01-30.09 2013	01.01-30.09 2012	01.01-31.12 2012
Ordinary profit before taxes		-6 562	-7 623	-18 715	-16 895	-25 259
Depreciation, Amortization and Write Off		-	-	-	17	17
Share options		-412	450	460	1 220	2 431
Net financials		-346	-444	-1 243	-1 657	-2 322
Changes in working capital		-495	-755	-2 689	-1 482	779
<b>Cash flow from operations</b>		<b>-7 815</b>	<b>-8 372</b>	<b>-22 187</b>	<b>-18 797</b>	<b>-24 354</b>
Net financials		346	444	1 243	1 657	2 322
Taxes paid		-	-	-	-	-
<b>Net cash flow from operations</b>		<b>-7 469</b>	<b>-7 928</b>	<b>-20 944</b>	<b>-17 140</b>	<b>-22 032</b>
<b>Cash flow from investments</b>						
Purchase of tangible assets		-	-	-	-	-
Purchase of intangible assets		-	-	-	-	-
<b>Net cash flow from investments</b>		<b>-</b>	<b>-</b>	<b>-</b>	<b>-</b>	<b>-</b>
<b>Cash flow from financial activities</b>						
Net proceeds from share issues		388	-	388	-	-
<b>Net cash flow from financial activities</b>		<b>388</b>	<b>-</b>	<b>388</b>	<b>-</b>	<b>-</b>
<b>Net change in cash during the period</b>		<b>-7 081</b>	<b>-7 928</b>	<b>-20 556</b>	<b>-17 140</b>	<b>-22 032</b>
Cash and cash equivalents at the beginning of the period		59 608	85 903	73 083	95 115	95 115
<b>Cash and cash equivalents at the end of the period</b>		<b>52 527</b>	<b>77 975</b>	<b>52 527</b>	<b>77 975</b>	<b>73 083</b>

## SELECTED EXPLANATORY NOTES:

### 1. Nature of operation

PCI Biotech Holding ASA (PCI Biotech) was established in 2008, and comprises PCI Biotech Holding ASA, the 100 percent 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 drug delivery technology for use in cancer therapy and other diseases. 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 drug delivery expert groups. PhotoChemical Internalisation (PCI) is a technology for light-directed drug delivery by triggered endosomal release and was developed to introduce therapeutic molecules in a biologically active form specifically into diseased cells.

The PCI technology has potential to improve the effect both of existing drugs and new classes of drugs, such as 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 technology and products for the delivery of marketed drugs and drugs in development. During the third quarter 2009, the first cancer patients received treatment in a Phase I/II trial with the patented lead candidate Amphinex in combination with the cytotoxic agent bleomycin. The trial was completed at University College Hospital (UCH) in London during Q2 2011. The study has primarily enrolled patients with Head & Neck cancer, a disease with local control issues that the PCI technology could potentially contribute to solve.

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 have been prepared in accordance with IAS 34 Interim Financial Statements and 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 28 October 2013.

### 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:

	Q3 2013	Q3 2012	9M 2013	9M 2012	FY 2012
Result allocated to shareholders (in NOK '000)	(6 562)	(7 623)	(18 715)	(16 895)	(25 259)
Weighted average of outstanding shares (in '000)	7 708	7 666	7 680	7 666	7 666
Earnings per share (NOK per share)	-0,85	-0,99	-2,44	-2,20	-3,29

Diluted earnings per share:

	Q3 2013	Q3 2012	9M 2013	9M 2012	FY 2012
Result allocated to shareholders (in NOK '000)	(6 562)	(7 623)	(18 715)	(16 895)	(25 259)
Weighted average of outstanding shares (in '000)	8 218	8 524	8 114	8 506	8 389
Earnings per share (NOK per share)	-0,85	-0,99	-2,44	-2,20	-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.

The Company's revenues are not influenced by any cyclicity of operations.

#### 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	Q3 2013	Q3 2012	9M 2013	9M 2012	FY 2012
Radiumhospitalets Forskningsstiftelse	550	780	1 719	1 169	1 593
Theresa Comiskey Olsen	-	-	3	3	3

At the end of the quarter, PCI Biotech had NOK 0.3 million in short term debt to 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 ongoing 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 30 September:

	Not due	Less than 3 months	3 to 12 months	Total
Trade receivables	-	-	-	-
Other receivables	6 290	-	-	6 290
<b>Total receivables</b>	<b>6 290</b>	<b>-</b>	<b>-</b>	<b>6 290</b>

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

#### Foreign currency risk

PCI Biotech has transactional currency exposure arising from sales and 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 Q4 2013.

### 8. Research and Development costs

	Q3 2013	Q3 2012	9M 2013	9M 2012	2012
Clinical studies	4 176	4 915	12 512	11 530	15 938
Pre-clinical studies	1 411	2 300	4 598	4 528	5 308
CMC and equipment	1 871	1 133	4 150	4 033	5 840
Patents	706	852	1 427	2 606	3 041
Other costs	0	0	0	0	1 135
<b>Total</b>	<b>8 164</b>	<b>9 200</b>	<b>22 687</b>	<b>22 697</b>	<b>31 262</b>

### 9. Deferred tax and deferred tax assets

At the end of the quarter, the company held NOK 39.5 million in non-capitalised deferred tax assets.

### 10. Share options

In Q2 2013, the following changes were 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 Q2 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% 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.

Due to the cancellation of share options there is a net cost reduction of NOK 0.5 million in Q3 2013 compared to share option costs of NOK 0.5 million in Q3 2012.

On 28<sup>th</sup> 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:

Expiry date	Exercise price	Number of shares	
	in NOK per share	30.09.2013	30.09.2012
2013 - Q3	19.02	170 000	255 000
2014 - Q3	6.47	174 000	234 000
2015 - Q3	37.24	95 000	115 000
2017 - Q3	37.02	86 500	135 000
2018 - Q2	19.63	85 000	0
<b>Total</b>		<b>610 500</b>	<b>739 000</b>

#### 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.