



November 7, 2017

## Prothena Reports Third Quarter 2017 Financial Results and Provides R&D Update

- Net cash used in operating and investing activities was \$18.3 million in the third quarter and \$94.8 million for the first nine months of 2017; quarter-end cash and restricted cash position of \$460.1 million, provides funding to advance diverse pipeline
- Presented new research at HFSA Annual Scientific Meeting on the important role of the cardiac biomarker NT-proBNP in AL amyloidosis
- R&D Day planned for Thursday, November 16<sup>th</sup> in New York, NY

DUBLIN, Ireland, Nov. 07, 2017 (GLOBE NEWSWIRE) -- Prothena Corporation plc (NASDAQ:PRTA), a late-stage clinical biotechnology company focused on the discovery, development and commercialization of novel protein immunotherapies, today reported financial results for the third quarter and first nine months of 2017. In addition, the Company provided an update on its R&D programs.

"As we look forward to topline results from our Phase 2b PRONTO study and Phase 3 VITAL study of NEOD001, we are focused on executing activities across our clinical, regulatory and commercial functions," said Gene Kinney, PhD, President and Chief Executive Officer of Prothena. "With results from the PRONTO study expected in the second quarter of 2018, we continue to generate new research on the important role of the cardiac biomarker NT-proBNP in AL amyloidosis. New data we presented in September demonstrated that aggregated light chain binds to cardiomyocytes, induces oxidative stress, and increases the expression of the oxidative response marker heme oxygenase-1, which in turn increases NT-proBNP secretion. These new insights into the unique regulation of NT-proBNP in AL amyloidosis offer further support that an amyloid targeting approach can provide cardiac benefit with the potential to translate into improved survival for patients with AL amyloidosis. Beyond NEOD001, our team continues to advance a diverse pipeline of first-in-class approaches for diseases that lack effective therapies including PRX002 and PRX004, as well as several new targets in discovery."

### Third Quarter 2017 and Recent Highlights:

- Presented research at the Heart Failure Society of America (HFSA) Annual Scientific Meeting that further supports the important role of the cardiac biomarker NT-proBNP in both the biology and clinical aspects of AL amyloidosis. Preclinical data presented in a moderated [poster talk](#) and [poster session](#) at the conference demonstrated the relationship between misfolded light chain toxicity to heart cells and production of NT-proBNP.
- Announced clinical results of a [Phase 1b multiple ascending dose study](#) in patients with psoriasis. Clinical data demonstrated occupancy and downregulation of CD146 following administration of PRX003 consistent with our previous Phase 1a single ascending dose study. However, the clinical results in this study did not meet the pre-specified criteria for evidence of a well-defined relationship between biological activity and meaningful clinical effects required to advance PRX003 into mid-stage clinical development for psoriasis or psoriatic arthritis as previously planned.

### Upcoming Research and Development Milestones

**NEOD001** is a monoclonal antibody for the potential treatment of AL amyloidosis:

- Topline results in the Phase 2b [PRONTO](#) study expected in the second quarter of 2018

**PRX002/RG7935** is a monoclonal antibody for the potential treatment of Parkinson's disease:

- The Phase 2 PASADENA study, initiated in the second quarter of 2017, continues to enroll patients with early Parkinson's disease

**PRX004** is a monoclonal antibody for the potential treatment of ATTR amyloidosis:

- Clinical development expected to begin by mid-2018

### Third Quarter and First Nine Months of 2017 Financial Results

Prothena reported a net loss of \$52.4 million and \$105.5 million for the third quarter and first nine months of 2017, respectively, as compared to a net loss of \$43.2 million and \$111.2 million for the third quarter and first nine months of 2016, respectively. Net loss per share for the third quarter and first nine months of 2017 was \$1.37 and \$2.82, respectively, as compared to a net loss per share of \$1.26 and \$3.25 for the third quarter and first nine months of 2016, respectively.

Prothena reported total revenue of \$0.2 million and \$27.3 million for the third quarter and first nine months of 2017, respectively, as compared to total revenue of \$0.3 million and \$0.9 million for the third quarter and first nine months of 2016, respectively. The increase in revenue for the first nine months of 2017 was primarily due to achievement of a clinical milestone from Roche of \$30.0 million (of which \$26.6 million was recognized as collaboration revenue and \$3.4 million was recognized as an offset to R&D expenses).

Research and development (R&D) expenses totaled \$41.3 million and \$101.0 million for the third quarter and first nine months of 2017, respectively, as compared to \$26.8 million and \$79.7 million for the third quarter and first nine months of 2016, respectively. The increase in R&D expenses for the third quarter of 2017 was primarily due to higher product manufacturing costs, and to a lesser extent higher personnel and clinical trial costs. The increase in R&D expenses for the first nine months of 2017 was primarily due to higher personnel costs, and to a lesser extent higher clinical trial and product manufacturing costs. R&D expenses included non-cash share-based compensation expense of \$2.8 million and \$7.9 million for the third quarter and first nine months of 2017, respectively, as compared to \$2.0 million and \$5.2 million for the third quarter and first nine months of 2016, respectively.

General and administrative (G&A) expenses totaled \$12.4 million and \$34.2 million for the third quarter and first nine months of 2017, respectively, as compared to \$16.1 million and \$31.5 million for third quarter and first nine months of 2016, respectively. The decrease in G&A expenses for the third quarter of 2017 compared to the same period in the prior year was primarily due to \$6.5 million of share-based compensation expense related to the accelerated vesting of stock options upon the passing of the Company's former CEO in the third quarter of 2016, offset in part by higher personnel costs in the third quarter of 2017. The higher G&A expenses for the first nine months of 2017 compared to the same period in the prior year was primarily due to higher personnel costs, and to a lesser extent higher consulting and other expenses, partially offset by a gain recognized from the assignment of the Company's former South San Francisco facility lease in January 2017 and the higher share-based compensation expense related to the accelerated vesting of stock options in the comparable period the prior year. G&A expenses included non-cash share-based compensation expense of \$4.3 million and \$11.5 million in the third quarter and first nine months of 2017, respectively, as compared to \$9.5 million and \$14.5 million in the third quarter and first nine months of 2016, respectively.

Total non-cash share-based compensation expense was \$7.1 million and \$19.4 million for the third quarter and first nine months of 2017, respectively, as compared to \$11.4 million and \$19.7 million for the third quarter and first nine months of 2016, respectively.

As of September 30, 2017, Prothena had \$460.1 million in cash, cash equivalents and restricted cash and no debt.

As of October 20, 2017, Prothena had approximately 38.4 million ordinary shares outstanding.

The Company is updating its projected full year 2017 net cash burn from operating and investing activities, and expects it to be \$142 to \$152 million, representing a decrease of approximately \$18 million based on a combination of savings related to the decision not to advance PRX003 and other favorability from development, manufacturing and taxes. The Company now expects to end the year with approximately \$409 million in cash, cash equivalents and restricted cash (midpoint). The cash increase of \$34 million results from the \$18 million in operating and investing burn savings described above and an additional \$16 million in financing proceeds from employee stock option exercises which occurred in the first three quarters of 2017 (primarily related to our former CEO's options, which were fully exercised by the end of the third quarter of 2017). The updated estimated full year 2017 net cash burn from operating and investing activities is primarily driven by the updated estimated net loss of \$150 to \$164 million, which includes an estimated \$27 million of non-cash share-based compensation expense.

### **Upcoming Investor Event**

Prothena will host an R&D Day on Thursday, November 16<sup>th</sup> from 12:00 - 2:00 PM in New York, NY. During R&D Day, Prothena management will discuss the Company's clinical development programs and highlight new discovery efforts.

A live webcast of the presentation can be accessed through the Investors section of the Company's website at [www.prothena.com](http://www.prothena.com). Following the live presentations, a replay of the webcast will be available on the Company's website for at least 90 days following the presentation date.

### **About Prothena**

Prothena Corporation plc is a global, late-stage clinical biotechnology company establishing fully-integrated research, development and commercial capabilities. Fueled by its deep scientific understanding built over decades of research in protein misfolding and cell adhesion - the root causes of many serious or currently untreatable amyloid and inflammatory diseases - Prothena seeks to fundamentally change the course of progressive diseases associated with this biology. The Company's pipeline of antibody therapeutic candidates targets a number of indications including AL amyloidosis (NEOD001), Parkinson's disease and other related synucleinopathies (PRX002/RG7935) and ATTR amyloidosis (PRX004). The Company continues discovery of additional novel therapeutic candidates where its deep scientific understanding of disease pathology can be leveraged. For more information, please visit the Company's website at [www.prothena.com](http://www.prothena.com)

## Forward-looking Statements

*This press release contains forward-looking statements. These statements relate to, among other things, whether an amyloid targeting approach can provide cardiac benefit with the potential to translate into improved survival for AL amyloidosis patients; the role of NT-proBNP in the biology and clinical aspects of AL amyloidosis; the relationship between misfolded light chain toxicity in heart cells and production of NT-proBNP; our ability to advance a diverse pipeline, including new targets in discovery; the timing of announcing topline results from the Phase 2b study of NEOD001; the timing of initiating clinical development of PRX004; our expected net cash burn from operating and investing activities for 2017 and cash balance at the end of 2017; and our estimated net loss and non-cash share-based compensation expense for 2017. These statements are based on estimates, projections and assumptions that may prove not to be accurate, and actual results could differ materially from those anticipated due to known and unknown risks, uncertainties and other factors, including but not limited to the risks, uncertainties and other factors described in the "Risk Factors" sections of our Annual Report on Form 10-K filed with the Securities and Exchange Commission (SEC) on February 27, 2017 and our subsequent Quarterly Reports on Form 10-Q filed with the SEC. Prothena undertakes no obligation to update publicly any forward-looking statements contained in this press release as a result of new information, future events or changes in Prothena's expectations.*

## PROTHENA CORPORATION PLC CONSOLIDATED STATEMENTS OF OPERATIONS (unaudited - amounts in thousands except per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2017	2016	2017	2016
Collaboration revenue	\$ 219	\$ 286	\$ 27,290	\$ 884
Total revenue	219	286	27,290	884
Operating expenses:				
Research and development	41,315	26,838	101,045	79,690
General and administrative	12,438	16,136	34,182	31,452
Total operating expenses	53,753	42,974	135,227	111,142
Loss from operations	(53,534)	(42,688)	(107,937)	(110,258)
Other expense, net	(565)	(130)	(2,195)	(156)
Loss before income taxes	(54,099)	(42,818)	(110,132)	(110,414)
Provision for (benefit from) income taxes	(1,705)	421	(4,653)	791
Net loss	\$ (52,394)	\$ (43,239)	\$ (105,479)	\$ (111,205)
Basic and diluted net loss per share	\$ (1.37)	\$ (1.26)	\$ (2.82)	\$ (3.25)
Shares used to compute basic and diluted net loss per share	38,292	34,413	37,384	34,266

## PROTHENA CORPORATION PLC CONSOLIDATED BALANCE SHEETS (unaudited - amounts in thousands)

	September 30, 2017	December 31, 2016
<b>Assets</b>		
Cash and cash equivalents	\$ 456,061	\$ 386,923
Other current assets	13,003	4,439
Total current assets	469,064	391,362
Property and equipment, net	55,384	56,452

Restricted cash	4,056	4,056
Other assets	9,176	8,106
Total non-current assets	68,616	68,614
Total assets	<u>\$ 537,680</u>	<u>\$ 459,976</u>
<b>Liabilities and Shareholders' Equity</b>		
Accrued research and development	\$ 18,482	\$ 19,073
Other current liabilities	20,776	22,002
Total current liabilities	39,258	41,075
Non-current liabilities:	52,168	53,498
Total liabilities	91,426	94,573
Total shareholders' equity	446,254	365,403
Total liabilities and shareholders' equity	<u>\$ 537,680</u>	<u>\$ 459,976</u>

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